

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

- (Mark One)
- ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the fiscal year ended December 31, 2020
- OR
- TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934 FOR THE TRANSITION PERIOD FROM** _____ **TO** _____

Commission File Number 001-39376

POSEIDA THERAPEUTICS, INC.

(Exact name of Registrant as specified in its Charter)

Delaware
(State or other jurisdiction of
incorporation or organization)
9390 Towne Centre Drive
San Diego, CA
(Address of principal executive offices)

47-2846548
(I.R.S. Employer
Identification No.)

92121
(Zip Code)

Registrant's telephone number, including area code: (858) 779-3100

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common stock, \$0.0001 par value per share	PSTX	Nasdaq Global Select Market

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the Registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. YES NO

Indicate by check mark if the Registrant is not required to file reports pursuant to Section 13 or 15(d) of the Act. YES NO

Indicate by check mark whether the Registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the Registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. YES NO

Indicate by check mark whether the Registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the Registrant was required to submit such files). YES NO

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
Emerging growth company	<input checked="" type="checkbox"/>		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report.

Indicate by check mark whether the Registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). YES NO

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the Registrant as of July 14, 2020 was approximately \$516.1 million, based on the closing price of the Registrant's common stock as reported by The Nasdaq Global Select Market on such date. The Registrant has elected to use July 14, 2020 as the calculation date, as this was the date the Company completed its initial public offering and on the last business day of the Registrant's most recently completed second fiscal quarter there was no public market for the Registrant's common stock.

The number of shares of Registrant's Common Stock outstanding as of March 5, 2021 was 62,108,168.

DOCUMENTS INCORPORATED BY REFERENCE

Certain information required to be disclosed in Part III of this report is incorporated by reference from the registrant's definitive Proxy Statement for the 2021 Annual Meeting of Stockholders, which proxy statement will be filed not later than 120 days after the end of the fiscal year covered by this report.

Table of Contents

	<u>Page</u>
PART I	
Item 1. Business	3
Item 1A. Risk Factors	72
Item 1B. Unresolved Staff Comments	126
Item 2. Properties	126
Item 3. Legal Proceedings	126
Item 4. Mine Safety Disclosures	126
PART II	
Item 5. Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities	127
Item 6. Selected Consolidated Financial Data	128
Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations	129
Item 7A. Quantitative and Qualitative Disclosures About Market Risk	142
Item 8. Financial Statements and Supplementary Data	142
Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure	142
Item 9A. Controls and Procedures	142
Item 9B. Other Information	143
PART III	
Item 10. Directors, Executive Officers and Corporate Governance	144
Item 11. Executive Compensation	144
Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters	144
Item 13. Certain Relationships and Related Transactions, and Director Independence	144
Item 14. Principal Accounting Fees and Services	144
PART IV	
Item 15. Exhibits, Financial Statement Schedules	145
Item 16. Form 10-K Summary	173

Forward-Looking Statements

This Annual Report includes forward-looking statements. All statements other than statements of historical facts contained in this Annual Report are forward-looking statements, including statements about:

- our expectations regarding the timing, scope and results of our development activities, including our ongoing and planned clinical trials;
- the timing of and plans for regulatory filings;
- our plans to obtain and maintain regulatory approvals of our product candidates in any of the indications for which we plan to develop them, and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- the potential benefits of our product candidates and technologies;
- our expectations regarding the use of our platform technologies to generate novel product candidates;
- the market opportunities for our product candidates and our ability to maximize those opportunities;
- our business strategies and goals;
- estimates of our expenses, capital requirements, any future revenue, and need for additional financing;
- our expectations regarding establishing manufacturing capabilities;
- the performance of our third-party suppliers and manufacturers;
- our expectations regarding our ability to obtain and maintain intellectual property protection for our platform technologies and product candidates and our ability to operate our business without infringing on the intellectual property rights of others;
- our expectations regarding developments and projections relating to our competitors, competing therapies that are or become available, and our industry;
- our expectations regarding the impact of the COVID-19 pandemic on our business, our industry and the economy;
- future changes in or impact of law and regulations in the United States and foreign countries; and
- the sufficiency of our existing cash, cash equivalents and short-term investments to fund our operations.

The words “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would” or the negative version of these words and similar expressions are intended to identify forward-looking statements. We have based these forward-looking statements on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, strategy, short- and long-term business operations and objectives and financial needs.

These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in the section titled “Risk Factors.” Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this Annual Report may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements

You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that the future results, advancements, discoveries, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur. Moreover, except as required by law, neither we nor any

other person assumes responsibility for the accuracy and completeness of the forward-looking statements. We undertake no obligation to update publicly any forward-looking statements for any reason after the date of this Annual Report to conform these statements to actual results or to changes in our expectations.

You should read this Annual Report and the documents that we reference in this Annual Report and have filed with the SEC with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

PART I

Item 1. Business.

We are a clinical-stage biopharmaceutical company dedicated to utilizing our proprietary gene engineering platform technologies to create next generation cell and gene therapeutics with the capacity to cure. We have discovered and are developing a broad portfolio of product candidates in a variety of indications based on our core proprietary platforms, including our non-viral piggyBac DNA Delivery System, Cas-CLOVER site-specific gene editing system and nanoparticle- and AAV-based gene delivery technologies. Our core platform technologies have utility, either alone or in combination, across many cell and gene therapeutic modalities and enable us to engineer our wholly-owned portfolio of product candidates that are designed to overcome the primary limitations of current generation cell and gene therapeutics.

Within cell therapy, we believe our technologies allow us to create product candidates with engineered cells that engraft in the patient's body and drive lasting durable responses that may have the capacity to result in single treatment cures. Our chimeric antigen receptor T cell, or CAR-T, therapy portfolio consists of both autologous and allogeneic, or off-the-shelf, product candidates. Relatively few allogeneic CAR-T cell products have been yet developed due to the need for a gene editing technology in their production, but these have the potential to be the next significant advance in the field as ready to use, off-the-shelf products of consistently high quality. We are advancing a broad pipeline with a plan to have up to six CAR-T product candidates in the clinic by 2022 in both hematological and solid tumor oncology indications. Our most advanced product candidate, P-BCMA-101, is an autologous CAR-T being developed for the treatment of patients with relapsed/refractory multiple myeloma, for which we have initiated a potentially registrational Phase 2 clinical trial, which we believe, if successfully completed, could support a submission seeking accelerated regulatory approval, and an expanded Phase 1 clinical trial that is currently enrolling to evaluate different dosing strategies and process improvements, including manufacturing using a nanoplasmid. In these clinical trials, following discussions with the U.S. Food and Drug Administration, or FDA, P-BCMA-101 can be dosed on a fully outpatient basis, without the need to reserve an intensive care unit, or ICU. The FDA has indicated that if data from our Phase 2 clinical trial do not provide evidence sufficient for accelerated approval, additional clinical testing would be required, including potentially a randomized controlled Phase 3 trial or trials. We are also currently enrolling patients in a Phase 1 clinical trial with our second autologous product candidate, P-PSMA-101, for the treatment of patients with metastatic castrate resistant prostate cancer, or mCRPC. We expect to file an investigational new drug application, or IND, for our first fully allogeneic CAR-T product candidate, P-BCMA-ALLO1, in the first half of 2021 for patients with relapsed/refractory multiple myeloma and have three additional allogeneic programs advancing toward anticipated IND filings in the second half of 2021 and 2022, including P-MUCIC-ALLO1 and up to two dual CAR allogeneic programs.

Within gene therapy, we believe our technologies have the potential to create next generation therapies that can deliver long-term, stable gene expression that does not diminish over time and may have the capacity to result in single treatment cures. We expect to file an IND for our first liver-directed gene therapy product candidate for the orphan genetic disease ornithine transcarbamylase, or OTC, deficiency in 2022. We believe our proprietary gene engineering technologies have the potential to address the limitations of the transient nature of traditional gene therapies, thereby offering distinct advantages in liver-directed gene therapy. Furthermore, we believe that we have the potential to pursue multiple *in vivo* and *ex vivo* approaches in a wide array of cell types and tissues for non-liver-directed gene therapies.

Across our pipeline, we seek to leverage the unique aspects and capabilities of our core platform technologies to create cell and gene therapeutic product candidates that: (1) are differentiated by potent and durable activity and

tolerability, (2) may allow us to address indications that are not accessible with the current generation of cell and gene therapeutics, and (3) may allow for widespread patient accessibility enabling broader commercial adoption.

Differentiation based on potent and durable activity and tolerability:

Cell Therapy. Our non-viral piggyBac DNA Delivery System allows us to design CAR-T product candidates that can not only deliver very large CAR-containing transgenes to T cells, but also generate CAR-T products that deliver a high percentage of early memory T cells, such as stem cell memory T, or T_{SCM}, cells. T_{SCM} cells are a stem cell form of T cells that engraft, self-renew and mature into every T cell subtype, including the effector T, or T_{EFF}, cells, which are tumor killing cells. We believe delivering a high percentage of T_{SCM} cells will drive more gradual tumor killing, thereby inducing less inflammatory cytokine response and improving the tolerability profile of our CAR-T product candidates relative to those of existing CAR-T therapies. Conceptually, through these T_{SCM} cells, we are able to deliver a predominantly self-renewing CAR-T “prodrug” that can engraft and produce unlimited T_{EFF} “drug”, an approach that potentially results in more potent activity and duration of response.

Gene Therapy. PiggyBac confers many potential advantages compared to current gene therapies that rely on traditional viral-based delivery methods. In preclinical studies, piggyBac transgene delivery exhibited high-level, long-term, stable gene expression and allowed for permanent gene integration into DNA. In contrast, traditional viral vectors used for *in vivo* gene therapy, such as adeno-associated virus, or AAV alone, which is a virus that can be engineered to deliver DNA to target cells, when used alone are unable to permanently integrate into DNA and thus result in transient therapeutic transgene expression, which decreases over time. PiggyBac’s ability to deliver high levels of stable integration and therapeutic transgene expression may also enable lower dosing when used in combination with AAV. Furthermore, in our preclinical studies the controlled integration of piggyBac has been shown to be non-mutagenic and non-oncogenic, which we believe makes it better suited as a delivery vehicle than AAV. As compared to nanoparticle alone-based delivery approaches, which similar to AAV alone approaches are transient in nature, nanoparticle combined with piggyBac may result in integration and stable therapeutic transgene expression and may also obviate the immunogenicity issues that are often associated with viral-based delivery methods.

Ability to address indications currently inaccessible by cell and gene therapeutics:

Cell Therapy. We believe the ability of our CAR-T product candidates to engraft and produce a potentially unlimited number of T_{EFF} cells is a critical advantage that may allow the field of CAR-T to move beyond hematological tumors and into solid tumors, an area historically limited due to the lack of persistence and durability of therapeutic cells needed to produce a clinical impact.

Gene Therapy. We are utilizing advantages that we have engineered in our piggyBac, nanoparticle and AAV-based gene delivery technologies to potentially overcome many of the limitations of current *in vivo* gene therapies. PiggyBac’s ability to permanently integrate into DNA enables us to extend our reach into diseases associated with many tissues of the body that contain either dividing or non-dividing cells, a feature not available to transient viral-based delivery methods. Additionally, our potential to enable durable gene expression within tissues with rapidly dividing cells should enable us to pursue the entire spectrum of genetic diseases including many indications within the pediatric population.

Widespread patient accessibility enabling broader commercial adoption:

Cell Therapy. CAR-T treatments have faced both cost and safety challenges. Our engineering of proprietary booster molecules allows us to generate hundreds of doses from a single manufacturing run in our fully allogeneic CAR-T program. We believe this will lead to a significant reduction in costs to levels in the range of traditional biologic therapeutics in oncology. Additionally, piggyBac is intrinsically more cost effective than historical CAR-T methods as it utilizes nucleic acids, DNA and RNA produced using good manufacturing practices, or GMP, which are faster and cheaper to produce than GMP virus. Our focus on T_{SCM}, first initiated in our autologous CAR-T product candidates, offers potential tolerability benefits and has demonstrated our potential ability to limit cytokine release syndrome, or CRS, and neurotoxicity that has limited the broad commercial adoption and utility of existing autologous CAR-T therapeutics. As a result of its tolerability profile and following discussions with the FDA, our

potentially registrational Phase 2 clinical trial can be dosed on a fully outpatient basis, which we believe will also support broader adoption, if approved.

Gene Therapy. PiggyBac's ability to permanently integrate into the DNA yields the potential to provide more durable responses within gene therapy for many diseases that current viral-based approaches are unable to address. Importantly, we believe piggyBac will drive our potential ability to deliver single treatment cures, overcoming the limitations of viral-based therapies related to tolerability and durability. PiggyBac in combination with AAV may enable lower dosing, thereby improving tolerability and reducing costs. In future product candidates, nanoparticle delivery of piggyBac will eliminate the need for AAV and may further improve tolerability and reduce cost. We believe these characteristics will potentially yield significant commercial advantages and confer meaningful pharmacoeconomic benefits to payors potentially resulting in broader commercial success.

Our Proprietary Cell and Gene Engineering Platform Technologies

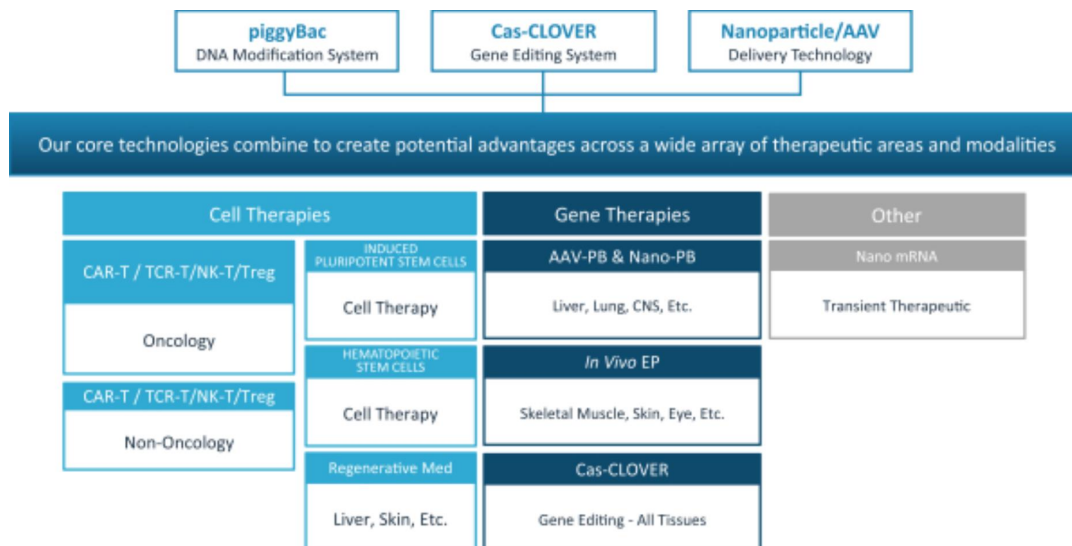
We have developed a proprietary suite of gene engineering technologies that have broad utility. The breadth and depth of our technology platforms fall into three primary categories: (1) non-viral gene insertion, (2) gene editing and (3) gene delivery, supported by CAR-T tools.

- **Non-viral gene insertion.** Our proprietary, non-viral piggyBac DNA Delivery System, which includes our Super piggyBac transposase enzyme, is highly efficient at stable gene insertion and has a significantly larger genetic cargo capacity as compared to viral methods (potentially greater than 20x lentivirus). As a result, our product candidates can contain transgenes large enough to include multiple chimeric antigen receptor, or CAR, and/or T cell receptor, or TCR, genes, selection genes, safety switch genes and potentially other cargo for specific treatment applications, making it a highly versatile platform. Importantly, piggyBac works in a wide variety of cell types, both dividing and non-dividing, T cells, B cells, natural killer cells, hematopoietic stem cells, or HSC, induced pluripotent stem cells, primary hepatocytes and numerous other cell types giving it broad reach and applicability.
- **Gene editing with precise specificity.** Our proprietary, highly precise Cas-CLOVER site-specific gene editing technology is easy to use, highly efficient and capable of multiplexing and has shown low to no off-target activity in our preclinical studies, which we believe provides a distinct tolerability advantage over other gene editing systems. In addition, unlike many other gene editing technologies, Cas-CLOVER can efficiently edit resting T cells, allowing for the maintenance of the highly desirable T_{SCM} product composition in allogeneic product candidates, an important component of our CAR-T approach. Both of our proprietary site-specific gene editing platforms, Cas-CLOVER, and a related technology called TAL-CLOVER, can also be used for *in vivo* gene therapies.
- **Gene delivery.** We have numerous technologies and platforms for delivering DNA, RNA and proteins, including into cells both *ex vivo* and *in vivo*. These include nanoparticle technology, AAV technology, and both *ex vivo* and *in vivo* electroporation, which is a process by which we use a pulse of electricity to briefly increase the permeability of cells.
- **Additional proprietary tools.** We also have a number of other technologies and tools that have been developed for certain specific applications including:
 - *T_{SCM} Phenotype.* We have developed and patented a number of manufacturing methods and media to preserve a high percentage of T_{SCM} in our product candidates. We believe that the T_{SCM} cell phenotype is key to success in CAR-T therapies.
 - *Positive selection.* We create product candidates utilizing a fully human drug resistance gene that can be employed during manufacturing to create a purified product that is essentially 100% CAR-positive, minimizing one of the sources of CAR-T toxicity and thereby potentially enhancing the therapeutic index. Our initial use for positive selection is for CAR-T, but this technology has utility in other cell types.
 - *Booster molecules.* We have developed a technology that enables improved expansion of gene-edited allogeneic cells without affecting their desirable T_{SCM} characteristics. The booster molecule is an RNA-based technology introduced to T cells during the manufacturing process, which results in transient expression of a receptor on the surface of T cells that allows the cells to respond to antibody-based activator molecules, resulting in significant expansion of the cells

without causing maturation or exhaustion of the cells. Using this approach, we can create hundreds of doses from a single manufacturing run yet maintain the high percentage of desirable T_{SCM} cells in the final product candidate. This technology is currently used in our allogeneic CAR-T program but may have utility in other cell types.

- *Safety switch.* We have developed a proprietary safety switch comprised of fully human genes that can be activated by administration of a small molecule, and thereafter, has the potential to rapidly eliminate some or all of the genetically modified cells in the patient after administration.
- *CAR binding libraries.* In addition to traditional scFv binders, we have access to and utilize novel binder technologies, such as heavy-chain-only antibody fragments, which, compared to scFv, are more stable, result in less T cell exhaustion and may result in lower immunogenicity.
- *Armoring platforms.* We can use our genetic engineering tools to make other modifications to our product candidates to potentially improve their performance against solid tumors, an approach commonly referred to as “armoring”. We have several types of armoring platforms:
 - *Conditional gene expression system:* Due to the very large cargo capacity of piggyBac, we have demonstrated the ability to deliver into the genome a conditional gene expression system that expresses one or more genes of interest only when the cell becomes activated or stimulated by binding of the CAR molecule to its specific target. This approach is superior to constitutive expression systems in that tight conditional regulation limits gene expression to relevant sites, such as the tumor microenvironment. In this way, supporting molecules such as pro/anti-inflammatory molecules, checkpoint inhibitors, cytokines, interleukins and chemokines can be expressed by the T cell and/or delivered locally to the tumor or target cell.
 - *Decoy receptors:* CAR-T therapies can be enhanced by using piggyBac to deliver molecules that sequester and block negative immune regulators, such as PD-1 and TGFβR2. Decoy/null or positive switch receptors can be used to block or convert to activators, respectively, regulatory signals from the tumor microenvironment that otherwise work to exhaust T cell responses.
 - *Gene knockout:* Our Cas-CLOVER site-specific gene editing platform can be used to armor both autologous and allogeneic CAR-T therapies by targeting functional regulatory molecules, such as checkpoint blockade genes. These protein receptors are involved in exhaustion mechanisms by the tumor microenvironment.

These broad platform technologies, when used in various combinations, enable us to pursue a wide array of therapeutic modalities and indications. We believe this component of our strategy and business model will be a core value driver for us over the long term. The following graphic presents the broad utility of our platform technologies:



Our Pipeline

Our broad and versatile set of proprietary platform technologies has allowed us to develop a deep pipeline of wholly-owned, novel product candidates with composition of matter protection through at least 2037. Our initial focus is on CAR-T for oncology and liver-directed gene therapy programs for rare diseases. The following table summarizes our current product candidate portfolio:

Indication	Candidate	Preclinical	IND-Enabling	Phase 1	Phase 2	Phase 3
CAR-T FOR ONCOLOGY						
MULTIPLE MYELOMA	P-BCMA-101	Auto				
	P-BCMA-ALLO1	Allo				
	Dual CAR (BCMA/CD19)	Allo				
PROSTATE CANCER	P-PSMA-101	Auto				
	P-PSMA-ALLO1	Allo				
SOLID TUMOR	P-MUC1C-ALLO1	Allo				
	Dual CAR (Undisclosed)	Allo				
B - CELL	Dual CAR (CD19/CD20)	Allo				
LIVER DIRECTED GENE THERAPIES						
ORNITHINE TRANSCARBAMYLASE DEFICIENCY (OTC)	P-OTC-101	GT				
HEMOPHILIA A	P-FVIII-101	GT				

CAR-T for Oncology

Autologous Programs

Our autologous CAR-T product candidates are developed using a patient's own cells to treat his or her disease. We believe our ability to develop product candidates with a high percentage of T_{SCM} cells may result in

improved tolerability and the potential to see more durable responses than the current generation of CAR-T therapeutics. We are also exploring novel dosing strategies in our autologous programs to more fully optimize clinical approaches. Importantly, all the learnings from our autologous programs are also being transferred and utilized to inform and improve our allogeneic programs.

P-BCMA-101

- Our most advanced product candidate, P-BCMA-101, is an autologous CAR-T targeting B cell maturation antigen, or BCMA. We are currently evaluating P-BCMA-101 in a potentially registrational Phase 2 clinical trial and expanded Phase 1 clinical trial for the treatment of patients with relapsed/refractory multiple myeloma in the outpatient setting.
- Interim results from our ongoing Phase 1 clinical trial of P-BCMA-101 are encouraging. We have seen favorable tolerability results with very low levels of CRS and almost no neurotoxicity. Based on the interim tolerability results observed in the Phase 1 clinical trial, we initiated our Phase 2 clinical trial on a fully outpatient basis.
- We presented an update of our Phase 1 data at the American Society of Hematology Annual Meeting in December 2020 highlighting some modifications we made to our manufacturing process using a nanoplasmid which translated into an increase in the depth and rate of responses at comparable doses to the earlier cohorts. We continue to evaluate the additional data generated using this process.
- The FDA granted Regenerative Medicine Advanced Therapy Designation in November 2018 and Orphan Drug Designation in May 2019 for the treatment of multiple myeloma.
- Manufactured using our non-viral piggyBac DNA Delivery System.

P-PSMA-101

- Autologous CAR-T product candidate targeting prostate-specific membrane antigen, or PSMA, being developed to treat patients with mCRPC.
- In preclinical studies, P-PSMA-101 has demonstrated elimination of tumor cells to undetectable levels in 100% of animals, with only one incidence of a relapse in the low dose cohort. These data were generated in a preclinical model of mCRPC in which immuno-deficient mice were implanted with solid tumors comprised of a human mCRPC cell line. To our knowledge based on published literature, no other product candidate has shown complete solid tumor elimination in this preclinical model. Should P-PSMA-101 show promising efficacy in the clinic, we would likely accelerate our allogeneic version of this product candidate, P-PSMA-ALLO1.
- We have initiated a Phase 1 clinical trial and dosed the first patient in May 2020. Following a patient death, the trial was placed on clinical hold in August 2020 which was subsequently lifted in November 2020. The Phase 1 trial resumed and is ongoing.
- Manufactured using our non-viral piggyBac DNA Delivery System.

Allogeneic Programs

Our fully allogeneic CAR-T product candidates are developed using well-characterized cells derived from a healthy donor as starting material with the goal of enabling treatment of potentially hundreds of patients from a single manufacturing run. Doses could be cryopreserved and stored at treatment centers for future off-the-shelf use.

P-BCMA-ALLO1

- Allogeneic CAR-T product candidate targeting BCMA, being developed to treat relapsed/refractory multiple myeloma patients.
- We have designed P-BCMA-ALLO1 to be fully allogeneic, with genetic edits to eliminate or reduce both host-vs-graft and graft-vs-host alloreactivity.

- We anticipate an IND filing and initiation of a Phase 1 clinical trial in the first half of 2021.
- Manufactured using our proprietary Cas-CLOVER site-specific gene editing technology to reduce or eliminate reactivity, in addition to our piggyBac DNA Delivery System for non-viral gene insertion and our booster molecule technology for manufacturing scalability.

P-MUC1C-ALLO1

- Allogeneic CAR-T product candidate in preclinical development for multiple solid tumor indications. We believe P-MUC1C-ALLO1 has the potential to treat a wide range of solid tumors derived from epithelial cells, such as breast, colorectal, lung, ovarian, pancreatic and renal cancers, as well as other cancers expressing a cancer-specific form of the Mucin 1 protein, or MUC1-C.
- P-MUC1C-ALLO1 was designed to leverage the learnings of our P-BCMA-ALLO1 program. We have designed P-MUC1C-ALLO1 to be fully allogeneic, with genetic edits to eliminate or reduce both host-vs-graft and graft-vs-host alloreactivity.
- We have demonstrated the elimination of tumor cells to undetectable levels in a preclinical model of ovarian cancer and two models of breast cancer, including a model of triple negative breast cancer in which immuno-deficient mice were implanted with a human metastatic breast cancer cell line.
- We anticipate an IND filing and initiation of a Phase 1 clinical trial by the end of 2021.
- Manufactured using our proprietary Cas-CLOVER site-specific gene editing technology to reduce or eliminate reactivity, in addition to our piggyBac DNA Delivery System for non-viral gene insertion and our booster molecule technology for manufacturing scalability.

P-PSMA-ALLO1

- Allogeneic CAR-T product candidate targeting PSMA being developed to treat patients with mCRPC.
- We have designed P-PSMA-ALLO1 to be fully allogeneic, with genetic edits to eliminate or reduce both host-vs-graft and graft-vs-host alloreactivity.
- We anticipate an IND filing after analyzing preliminary results observed in the ongoing P-PSMA-101 Phase 1 clinical trial.
- Manufactured using our proprietary Cas-CLOVER site-specific gene editing technology to reduce or eliminate reactivity, in addition to our piggyBac DNA Delivery System for non-viral gene insertion and our booster molecule technology for manufacturing scalability.

Dual CAR Allogeneic Programs

We have a portfolio of allogeneic dual CAR product candidates, which contain two fully functional CAR molecules to target cells that express at least one of the two intended targets, that are in preclinical studies. We believe that our ability to include two or more fully functional CAR molecules into a T cell could be a significant competitive advantage. We intend to file an IND and initiate a Phase 1 clinical trial for at least one dual CAR program in 2022.

Gene Therapy Programs

Our gene therapy product candidates have been developed by utilizing our piggyBac technology together with AAV to overcome the major limitations of traditional AAV gene therapy. We believe that our approach will result in integration and long-term stable expression at potentially much lower doses than AAV technology alone, thus also conferring cost and tolerability benefits. Our eventual goal is to completely replace AAV with our nanoparticle technology, freeing future product development in gene therapy of AAV limitations.

P-OTC-101. P-OTC-101 is a liver-directed gene therapy combining piggyBac technology with AAV for the *in vivo* treatment of OTC deficiency. OTC deficiency is an often fatal or morbid urea cycle disease caused by congenital mutations in the OTC gene with a high unmet medical need. In a neonatal mouse model of severe OTC

deficiency, we observed an approximately 100-fold increase in OTC levels with a single injection of a piggyBac in combination with AAV to deliver an OTC therapeutic transgene compared to AAV-transgene alone, significantly beyond what would be expected to correct the deficiency in humans. We anticipate an IND filing and initiation of a Phase 1 clinical trial for P-OTC-101 in 2022.

P-FVIII-101. P-FVIII-101 is a liver-directed gene therapy combining piggyBac technology with our nanoparticle delivery technology for the *in vivo* treatment of Hemophilia A. Hemophilia A is a bleeding disorder caused by a deficiency in Factor VIII production with a high unmet need. Our preclinical data demonstrates an ability to correct this deficiency to near normal levels in a juvenile mouse model using nanoparticle delivery of our P-FVIII-101 potential product candidate. Preclinical studies are ongoing that will inform the development plan and timeline to IND.

P-MMUT-101. P-MMUT-101 is a liver-directed gene therapy combining piggyBac technology with AAV for the treatment of methylmalonic acidemia, or MMA. MMA is an inborn error of metabolism caused by congenital mutations in the methylmalonyl-CoA mutase, or MMUT, gene affecting amino acid metabolism pathways with a high unmet medical need. Due to uncertainty associated with the pandemic and the emergence of our Factor VIII program we will assess timing of this program following more data on nanoparticle delivery.

Our Strategy

Our mission is to develop next generation cell and gene therapeutics with the capacity to cure.

We intend to develop and commercialize novel cell and gene therapy products by using our broad gene engineering platform technologies to treat patients with high unmet medical need across a wide array of indications. Our current pipeline includes autologous and allogeneic CAR-T product candidates for oncology indications and piggyBac + AAV and piggyBac + nanoparticle product candidates as liver-directed gene therapy programs for orphan genetic diseases. We plan to pursue our mission through the following strategies:

Rapidly develop and commercialize autologous and allogeneic CAR-T therapies targeting hematological malignancies. We are developing both P-BCMA-101 and P-BCMA-ALLO1, product candidates for patients with relapsed/refractory multiple myeloma, to address cost and safety limitations of current CAR-T therapies utilized in this indication. Over time, we plan to develop our product candidates in earlier lines of treatment and for other hematological malignancies and will seek to commercialize in community hospital settings, and eventually in outpatient infusion sites. Based on the toxicity profile observed in the Phase 1 clinical trial and following discussions with the FDA, our potentially registrational Phase 2 clinical trial can be dosed on a fully outpatient basis. Should data from our P-BCMA-ALLO1 program, which we anticipate will begin to be available in late 2021, meet our expectations, we would consider reallocating our efforts and resources to advancing our allogeneic program.

Leverage the strength and breadth of our platform technologies to develop autologous and allogeneic CAR-T therapies in solid tumors. Our platform technology is designed to address the historical CAR-T limitations in treating solid tumors, which result from the lack of product persistence needed to have a clinical impact on these indications. We are advancing both P-PSMA-101, P-PSMA-ALLO1 and P-MUC1C-ALLO1 as candidates for the treatment of solid tumors. P-PSMA-101 is an autologous CAR-T being evaluated in a Phase 1 clinical trial in which patient dosing was initiated in May 2020 and enrollment resumed in late 2020 after a clinical hold from August to November 2020 following a patient death. Should P-PSMA-101 show promising efficacy in the clinic, we would likely accelerate our allogeneic version of this product candidate, P-PSMA-ALLO1. Due to the promising preclinical data we are seeing from P-BCMA-ALLO1, we have decided to advance our first CAR-T targeting MUC1-C, P-MUC1C-ALLO1, as a fully allogeneic program with the IND and start of a Phase 1 clinical trial expected by the end of 2021.

Utilize our platform technologies to pursue liver-directed gene therapy programs. Our lead gene therapy product candidates, P-OTC-101, P-FVIII-101 and potentially P-MMUT-101, utilize our piggyBac technology combined with AAV or nanoparticles to target orphan genetic diseases with the goal of developing single-treatment cures. Over time, we intend to develop additional therapies for rare diseases and to replace AAV technology with nanoparticle-based delivery of our *in vivo* gene therapies. We believe that nanoparticle delivery of gene therapy

would be a major advancement over AAV delivery by improving tolerability, lowering cost, allowing for re-dosing and addressing indications that AAV will not be able to effectively address, including diseases where correction necessitates delivery of large therapeutic transgenes. We plan to rapidly develop, and if approved, commercialize these gene therapy product candidates.

Utilize our technology and capabilities to develop allogeneic multi-CAR-T products. Our allogeneic product candidates include Dual CD19/CD20 for B cell malignancies and potentially some autoimmune diseases, Dual BCMA/CD19 for multiple myeloma and an undisclosed Dual CAR for solid tumors. We believe these multi-CAR programs highlight the ability of our piggyBac platform to enable product candidates that other technologies will not be able to achieve easily, if at all. We plan to continue developing multi-CAR product candidates, which we believe could represent a next generation of CAR-T therapies.

Evaluate strategic partnerships and structures to create value and continue to innovate and develop our platform technologies. Our platform technologies are highly differentiated with the ability to create many product candidates across a wide array of therapeutic modalities and indications. As such, we intend to seek partnerships and collaborations to expand our reach and create additional value in pursuit of our mission. In addition, we may evolve our corporate structure to implement a holding company or similar structure in order to maximize the value of our platform technologies and product candidates.

Our Team

We are led by an experienced management team with an unwavering commitment to developing next generation cell and gene therapeutics with the capacity to cure. Our Chief Executive Officer, Eric Ostertag, M.D., Ph.D., was the first graduate from the Gene Therapy Program at the University of Pennsylvania and has over 20 years of experience in cell and gene engineering, founding multiple biotechnology companies, including Transposagen Biopharmaceuticals. Dr. Ostertag served as Transposagen's Chief Executive Officer for 13 years, developing next-generation gene engineering technologies that were eventually spun out to create Poseida Therapeutics, in early 2015, and has served as our CEO since our founding. Our President and Chief Business Officer, Mark J. Gergen, J.D., has over 25 years of experience in healthcare and life science companies and, prior to joining our company in early 2018, was part of the executive management team for a number of successful biotechnology companies, including Amylin Pharmaceuticals, Mirati Therapeutics, and Halozyne Therapeutics. As of December 31, 2020, the management team is supported by our 206 employees, 110 of whom hold advanced degrees, including 54 with a Ph.D. and/or M.D. degree, and many with extensive experience in drug discovery and development.

Our Proprietary Platform Technologies

We believe we are well-positioned to drive the continued advancement of CAR-T therapies for the treatment of oncology indications, as well as gene therapies for severe orphan genetic diseases with the mission to create next generation product candidates with the capacity to cure. We have developed our genetic engineering technologies to overcome the primary limitations of current generation cell and gene therapeutics. Our technologies are highly differentiated and designed to provide potentially significant advantages as highlighted below:

- PiggyBac DNA Delivery System – advantages in cell therapy applications
 - Preferentially delivers therapeutic transgenes to T_{SCM} cells
 - Works in resting T cells, which is important in preserving T_{SCM} cells
 - Very large cargo capacity allows insertion of additional molecules, including multi-CAR and/or TCR approaches
- PiggyBac DNA Delivery System – advantages for in vivo gene therapy applications
 - Permanent and stable therapeutic transgene integration into DNA
 - Works efficiently in dividing and non-dividing cells and tissues
 - Potential to address pediatric liver indications

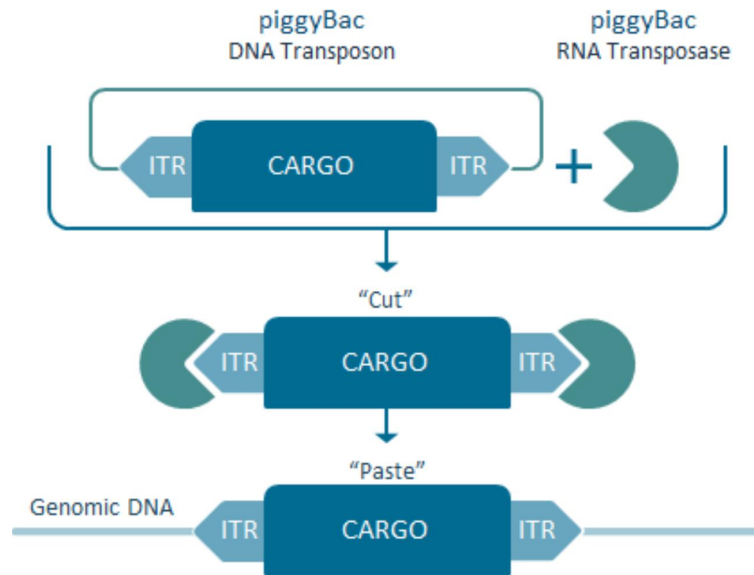
- May enable single-treatment cures
- Cas-CLOVER Site-Specific Gene Editing – advantages in cell therapy applications
 - Ability to perform highly efficient multiplexed gene editing enables fully allogeneic CAR-T product candidates
 - Efficient editing in resting T cells, which is important in preserving T_{SCM} phenotype in CAR-T
 - Precise gene editing: high on-target site specificity with no to very low off target activity minimizes tolerability concerns
 - Cas-CLOVER Site-Specific Gene Editing – advantages in gene therapy applications
 - Enables in vivo gene editing
 - Works in all types of cells and tissues tested to date
- Nanoparticle / AAV Delivery Technologies
 - Enables both ex vivo and in vivo gene therapies
 - Delivers piggyBac and Cas-CLOVER Systems to nearly any cell type or tissue
- Proprietary Tools, such as Booster Molecules
 - Booster molecules overcome the “Allo Tax”, which commonly refers to the suboptimal manufacturing yield and characteristics of CAR-T products due to genetic modification, by enabling improved expansion of genetically modified T cells
 - Enables expansion of T cells without affecting their desirable T_{SCM} characteristics
 - Allows us to create hundreds of doses from a single healthy donor

Our technology platforms fall into three primary categories: (1) non-viral gene insertion, (2) gene editing and (3) gene delivery, supported by CAR-T tools; and have broad utility and serve as the foundation of our development programs.

Non-viral gene insertion: piggyBac DNA Delivery System

DNA transposons are genetic elements that efficiently move from a plasmid to a chromosome via a cut and paste mechanism. DNA transposons have been used as a gene transfer method, including in CAR-T manufacturing. The piggyBac DNA Delivery System is our proprietary non-viral gene engineering technology that can be used to add therapeutic transgene DNA to the genome using the highly efficient Super piggyBac transposase enzyme, a hyperactive enzyme that was genetically modified to enable very high efficiency transposition of piggyBac transposons. We believe piggyBac enables efficient and precise transposition and multiple differentiated product attributes.

The image below depicts the piggyBac DNA Delivery System:



Therapeutic genes encoded within the cargo region of the piggyBac DNA transposon transgene are flanked by non-translated inverted terminal repeat sequences, or ITRs, that are specifically recognized by the transposase enzyme for the highly efficient process of stably integrating the therapeutic transgene cargo into specific sequences (TTAA nucleotides) in the genome. The transposase enzyme can be co-delivered to the cell as a protein or encoded in either DNA or RNA.

The piggyBac platform is our core technology used for the development of CAR-T and other gene therapy product candidates in our pipeline. We believe our piggyBac DNA Delivery System enables multiple differentiated product attributes including:

- CAR-T product candidates with a high percentage of desirable T_{SCM} cells, leading to better engraftment and duration of response with the potential for re-response, as well as a better tolerability profile;
- very large cargo capacity (potentially greater than 20x lentivirus)—allows efficient delivery of large therapeutic transgenes, including the possibility of multiple CAR or TCR molecules and incorporation of selection genes, safety switches and/or armoring strategies;
- non-viral delivery system that reduces the risk of mutagenesis and oncogenesis compared to viral delivery systems;
- high insertion efficiency and stable therapeutic transgene expression in a wide range of dividing and non-dividing cells and tissues; and
- shorter timelines and less costly manufacturing than viral methods.

As discussed previously, the piggyBac transposon preferentially transposes therapeutic transgenes into early memory T cells, including T_{SCM} cells. We believe retroviral transgene delivery methods, such as lentivirus and γ -retrovirus, are not efficient at delivering transgenes into early memory T cells. This is a key differentiator that allows us to manufacture CAR-T products with a high percentage of T_{SCM} cells, giving them desirable characteristics.

While the genetic cargo capacity of viruses typically used in CAR-T manufacturing, such as lentivirus and γ -retrovirus, is limited to approximately 10-20 kilobases, or kb, piggyBac has demonstrated cargo delivery of greater than 200 kb, allowing transfer of multiple useful genes. The very large cargo capacity of piggyBac permits

incorporation of multiple genes into our product candidates to further enhance tolerability and potency, with all CAR-T cells in our current CAR-T product candidates carrying a CAR molecule gene, a safety switch gene and a selection gene. The cargo capacity also allows for packaging of multiple CAR-T encoding genes and/or TCR genes allowing for the creation of dual and other multi-CAR-T product candidates.

PiggyBac ITRs and other components act as strong insulators, ensuring stable transgene expression and reducing risks of oncogenesis. PiggyBac has shown lower integration into intragenic regions compared with lentivirus, meaning that it is less likely to cause a detrimental mutation.

Additionally, piggyBac is estimated to have a significantly lower cost in production of GMP material and a much shorter timeline for GMP production as compared to GMP production of viral vectors.

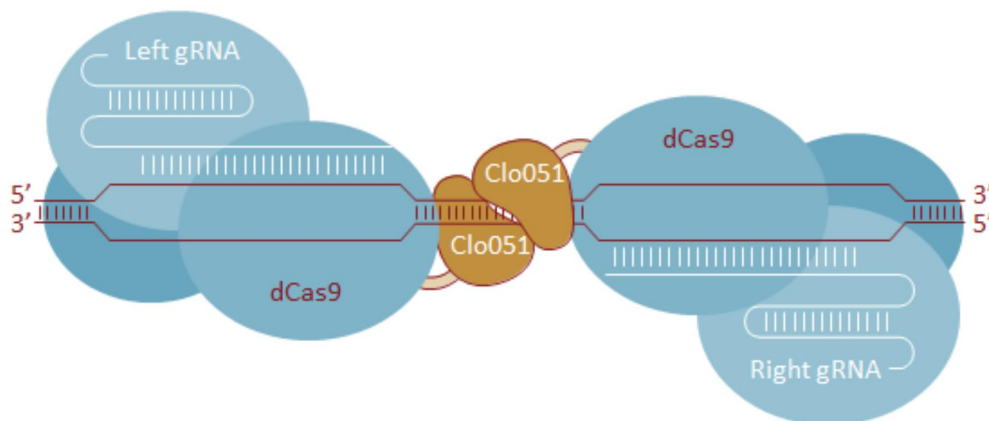
The image below depicts our piggyBac transposon transgene approach for creating CAR-T product candidates:



Gene Editing with Precise Specificity: Cas-CLOVER Site-Specific Gene Editing Technology

We have developed gene editing technology that uses a proprietary obligate homodimer nuclease system named CLOVER, which consists of parts of the Type IIS restriction endonuclease, Clo051. Genome cutting by this enzyme is strictly dependent upon dimerization, which makes it a fully dimeric system and gives it precise site-specificity. Cas-CLOVER uses a CRISPR (Clustered, Regularly Interspaced Short Palindromic Repeats) associated protein 9, or Cas9, enzyme that has been permanently altered and is unable to cut DNA (called dCas9). The dCas9 acts only as a DNA binding protein when combined with an appropriate guide RNA (gRNA). Cas-CLOVER combines the advantages of the first-generation CRISPR system (ease of design, low cost, multiplexing ability) with the advantages of the obligate homodimer nuclease systems (precise specificity). Importantly for T cell applications, Cas-CLOVER works well in resting T cells, which allows us to avoid maturation and exhaustion during production and assists in preserving the T_{SCM} phenotype.

Cas-CLOVER Gene Editing System



The most widely used platform for gene editing is CRISPR and an associated protein, Cas9. This gene editing technology is derived from a naturally occurring viral defense mechanism in bacteria. It works by binding the Cas9 enzyme to guide RNA, which can direct the Cas9 enzyme to a specific DNA sequence to make cuts in double-stranded DNA. Once the DNA is cut, the cell uses naturally occurring DNA repair mechanisms to rejoin the cut ends.

The CRISPR/Cas9 technology has been shown to result in unwanted off-target cutting, which means additional cutting at unintended sites that are often similar but not identical to the target DNA site. This off-target cutting can result in permanent mutations to the genomic DNA, which may unintentionally lead to detrimental mutations and oncogenesis, thereby creating significant safety concerns when used for the manufacture of cell and gene therapeutics.

Another popular site-specific gene editing platform used for cell and gene therapeutic applications are the Transcription Activator-Like Effector Nucleases, or TALENs. They are constructed by fusing a TAL DNA-binding domain to a DNA cleavage domain, typically FokI, which functions as an obligate homodimer, meaning two half-sites must come together at the exact same place and the exact same time in order to make a cut. Given the requirement for two half-sites, this type of system is sometimes called a fully dimeric system.

While TALEN technology can often cut specific sites in DNA with much higher fidelity than CRISPR/Cas9, it is relatively labor intensive and expensive to build. Conceptually similar, ZFN technology is a gene editing technology comprised of a class of DNA binding proteins used to make double-stranded breaks in DNA. Like TALEN technology, ZFN requires more preparation and work to use through the creation of arrays needed to target specific desired edits. TALEN and ZFN technologies both require activation of the cells to edit and do not work well in resting T-cells, and thus fail to preserve a high percentage of the T_{SCM} phenotype for CAR-T.

Another emerging gene editing technology is known as base editors. Base editing uses components from CRISPR systems together with other enzymes to directly install point mutations into cellular DNA or RNA without making double-stranded DNA breaks. DNA base editors comprise a catalytically disabled nuclease fused to a nucleobase deaminase enzyme and, in some cases, a DNA glycosylase inhibitor. Base editing technology is known to create some level of unwanted off-target mutations but the full extent is not yet known and could present a safety concern for allogeneic CAR-T where products could be given to many patients.

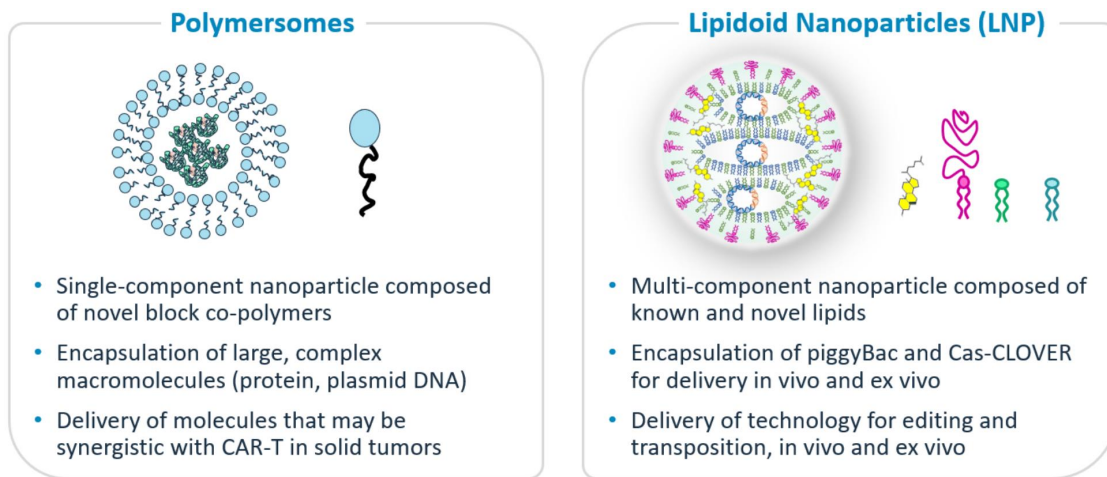
Gene Delivery Technologies: Nanoparticle Technology, In vivo and Ex vivo Electroporation and AAV

In addition to our piggyBac platform for non-viral gene insertion and our Cas-CLOVER platform for gene editing, we have developed a set of platform technologies for gene delivery to allow us to deliver RNA, DNA and proteins into cells both *ex vivo* and *in vivo* for various applications. These technologies include nanoparticle technology, AAV technology and *ex vivo* and *in vivo* electroporation technologies and approaches. Because of the breadth of potential utility of piggyBac and Cas-CLOVER, we foresee a need for different delivery modalities for different applications.

In our autologous and allogeneic CAR-T product candidates, we edit the T cells *ex vivo* using electroporation to deliver the necessary piggyBac components required to stably insert the therapeutic transgene into the genome of the cells. In the case of our allogeneic CAR-T product candidates, we also introduce Cas-CLOVER into the T cells via electroporation to edit the cells to eliminate alloreactivity. In our initial liver-directed gene therapy program, we are currently using AAV technology to deliver piggyBac to the liver *in vivo*.

We have developed a variety of distinct nanoparticle compositions to achieve different delivery objectives. These nanoparticles fall generally into two categories, polymersomes and lipid nanoparticles, or LNPs. Polymersomes are single component particles comprised of novel block co-polymers and are designed to deliver large complex molecules such as proteins. LNPs are multi-component nanoparticles composed of known and novel lipids and are designed to deliver nucleic acids including mRNA and DNA. We are evaluating polymersomes to

deliver therapeutic proteins that may be synergistic with our solid tumor CAR-T product candidates. We are evaluating LNPs to deliver both our piggyBac and Cas-CLOVER technologies.



Our longer-term goal for our nanoparticle platform is to be able to eliminate the need for AAV for *in vivo* gene therapies or *ex vivo* electroporation for *ex vivo* gene therapies by using nanoparticles to deliver our technologies into cells. We are also developing the technology and ability to deliver piggyBac and Cas-CLOVER through *in vivo* electroporation. While we have not yet nominated a product candidate using *in vivo* electroporation technology, we are exploring delivery of therapies to tissues that can be accessed from outside the body including skin, muscle and eye, which could open a range of potential development areas and new programs.

CAR-T for Oncology: History of CAR-T

Until recently, all major treatment modalities for cancer shared the same problem: they killed cancer cells, but not without damaging healthy cells and tissues. Immuno-oncology, the concept of using the patient's own immune system to attack cancer, has the potential to eliminate this challenge. A person's adaptive immune system is responsible for recognizing and eliminating a number of threats to the body, such as infectious agents, as well as infected and abnormal cells. T cells, specialized white blood cells capable of detecting and killing infected and abnormal cells, are a crucial component of this adaptive immune response. CAR-T therapies work to redirect these T cells, which are extremely specific killers, to kill cancer cells through genetic modification.

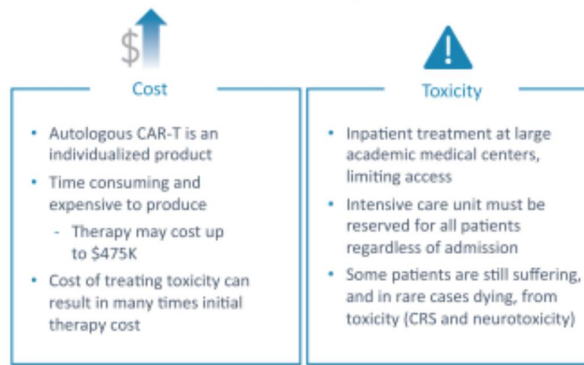
CAR-T therapy has emerged as a revolutionary and potentially curative therapy for patients with hematologic cancers, including those that have become heavily refractory to standard therapy. Currently, only autologous CAR-T therapy is available, in which T cells are removed from the body, engineered with receptors specific to cell surface targets on the patient's tumor cells, and administered back into the body. Once the engineered T cells are administered, they are able to recognize and kill the tumor cells that express the target for the engineered receptor. Researchers are now also developing allogeneic, or off-the-shelf, CAR-T therapy, in which a single donor or cell line is used to create a large number of doses of CAR-T, thereby greatly reducing the costs of manufacturing.

The Challenges to Widespread Adoption of CAR-T

Despite the potent activity from early CAR-T entrants to the market, commercial adoption has been relatively slow to date. We believe that there are two main hurdles to widespread adoption of CAR-T. The first hurdle is cost. The therapies themselves can cost hundreds of thousands of dollars, and there are potentially significant additional costs from managing the occasionally substantial toxicities from the early-generation CAR-T therapies. The second hurdle is the toxicities themselves. While some progress is being made in managing the side effects, the risk remains significant for many patients, requiring that these early generation CAR-T products to be administered only in large

hospitals and treatment centers with intensive care units, as compared to more accessible community hospitals and outpatient infusion centers.

The Two Main Hurdles for Widespread Adoption of CAR-T



We believe that our approach enables us to address these hurdles to unlock the potential of CAR-T therapies. The combination of our higher percentage T_{SCM} product and a potentially improved tolerability profile may allow us to move beyond academic medical centers and broaden the reach of these products. In our ongoing clinical trials of P-BCMA-101, we are already dosing on a fully outpatient basis, following discussions with the FDA, which will enable expanded reach and lower cost. In addition, our booster molecule technology allows us to drive scale to our allogeneic manufacturing process, resulting from the ability to produce hundreds of doses of our allogeneic CAR-T product candidates from a single manufacturing run from a single healthy donor. This dramatically reduces the manufacturing cost of CAR-T therapy to levels in the range of traditional biologic therapeutics in oncology and enabling off-the-shelf availability for immediate use.



Addressing the Limitations of Early-Generation CAR-T Therapies

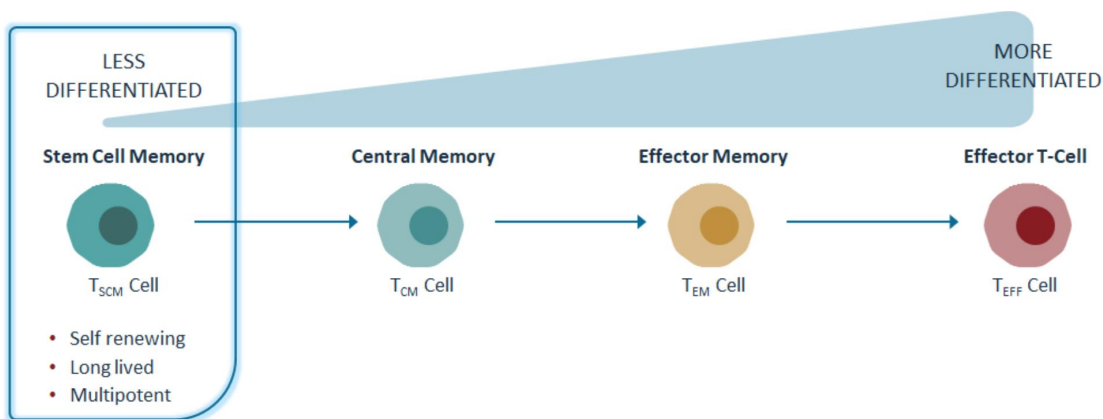
Although early-generation CAR-T therapy has shown significant potential, there are a number of limitations. The great majority of early-generation and current CAR-T therapies are produced using viral-based manufacturing. We believe that there are a number of inherent problems related to viral-based manufacturing that cause the limitations of other CAR-T therapies. T cell engineering is typically achieved via viral transduction, the process of introducing foreign DNA into a cell using a virus, most notably with retroviruses, such as γ-retrovirus or lentivirus.

Despite extensive optimization of these viral vectors, their limitations are becoming more evident, including safety concerns regarding the insertional profile, limited genetic cargo capacity, and undesirable characteristics of the final product. We use our proprietary non-viral piggyBac DNA Delivery System to deliver CAR molecule genes to T cells. The most significant advantage of using a non-viral approach is the ability to generate CAR-T products comprised of a high percentage of T_{SCM} cells. We believe this has the potential to result in therapies that elicit more consistent and durable responses with less toxicity. Additionally, we believe our non-viral approach will have much lower manufacturing costs and shorter manufacturing timelines. We also believe that our technology will enable us to develop allogeneic, or off-the-shelf, CAR-T therapies from healthy donors that will be potentially as good as or better than autologous CAR-T products, be available off-the-shelf and be a fraction of the cost of autologous therapies.

Not all T cells are created equally

T_{SCM} cells are believed to be ideal for cell therapy because they have the potential to engraft, be long-lived, self-renewing and multi-potent in that they can create wave after wave of more differentiated cells. There is a one-way maturation pathway from T_{SCM} cells to central memory T cells, or T_{CM}; then to effector memory T cells, or T_{EM}; and lastly, to T_{EFF} cells. As T cells mature and differentiate, their core functions and capabilities change, impacting their potency and durability. Our approach is to utilize a high percentage of less differentiated T cells in our product candidates with the goal of increasing persistence and mitigating some of the key limitations of early-generation CAR-T products. We also believe that creating a product with high T_{SCM} may potentially be the key to success in solid tumors where the T_{SCM} cells can engraft and create wave after wave of cells to attack the tumor. Conceptually, products that are more matured and contain more effector cells are like a drug, whereas our products that have a high percentage of T_{SCM} cells are like a prodrug. The T_{SCM} cells do not kill tumor cells, they engraft and create the more differentiated cells that do the killing.

The following figure illustrates this one-way T cell maturation pathway, from T_{SCM} cell to T_{EFF} cell:



Based upon our clinical data to date, we have observed a strong correlation between the percentage of T_{SCM} in the product candidate and best clinical response. In addition to our own experience, there is growing evidence and recognition that T_{SCM} is correlated with efficacy in the clinic.

CAR-T in Hematological Tumors

Early-generation CAR-T therapeutics have demonstrated an ability to achieve impressive responses in hematological malignancies, even in pre-treated patients who are relapsed and/or refractory to prior lines of standard therapies. Dramatically higher response rates than those reported for all prior therapeutics have been achieved in some indications, with some patients likely being cured. Despite these outcomes, however, significant challenges remain with regard to safety and cost. Furthermore, we believe additional improvements could be made with regard to duration of response as a number of patients have relapsed after receiving CAR-T therapy and duration of response has generally been poor.

A major limitation of early-generation CAR-T therapies is the potential for severe toxicity, most notably CRS and neurotoxicity, either of which can be fatal. Current CAR-T therapeutics are administered at large medical centers with ICUs so that an ICU can be reserved for all patients being administered CAR-T in the case they experience these severe toxicities. Furthermore, the cost of dealing with the toxicities associated with CAR-T can oftentimes exceed the cost of the therapeutic itself. There are also significant cost, manufacturing and commercial scalability challenges ahead for other CAR-T candidates, mainly due to the nature of viral-based manufacturing. These issues greatly limit the commercial reach of current CAR-T products. There are several potential reasons for the poor duration of response, which generally fall into two categories: elimination of the CAR-T cells from the body and loss of expression of a CAR-T target on a tumor cell, known as antigen escape.

Safety

The excitement over the impressive responses seen initially with early-generation CAR-T approaches has unfortunately been tempered by potentially life-threatening toxicities, most notably CRS and neurotoxicity. Typical clinical symptoms of neurotoxicity include headache, confusion, delirium, language disturbance and seizures. As more is being understood about these toxicities, it is now appreciated that they may be caused by different molecular mechanisms. However, both are rooted in a T cell response that is essentially too rapid and too strong. The CAR-T cells and other immune cells of the patient release cytokines and other molecules that initiate immune cascades that can be fatal if not avoided or successfully treated.

T_{SCM} cells express fewer cytotoxic effector molecules than more matured T cells and are postulated to differentiate and develop cytotoxic capability gradually. We believe the T_{SCM} cell phenotype may lead to a more controlled expansion of CAR-T and more gradual killing of tumor cells, thereby lessening the severity of toxicities, such as CRS and neurotoxicity, and resulting in a CAR-T product that can be administered on a fully outpatient basis.

A second safety feature incorporated into our CAR-T product candidates is the positive selection for CAR-positive cells during the manufacturing process. Drug resistance genes have been employed in other cellular therapeutics as a mechanism for selecting and purifying gene-modified cells to improve the efficiency of gene therapy. Our product candidates are engineered to express a variant of the human dihydrofolate reductase, or DHFR, gene. Cells containing this variant of the DHFR gene are slightly resistant to the drug methotrexate, or MTX. The advantage of DHFR over other drug-resistance strategies is that MTX is not genotoxic and preferentially kills dividing cells. Importantly, this gene-drug combination has been previously demonstrated to permit *ex vivo* selection of genetically modified T cells with relatively low concentrations of MTX.

Additionally, we enrich for gene-modified CAR-positive cells during *ex vivo* expansion, thereby purifying the therapeutic product and controlling for any patient-to-patient variability in raw material or manufacture, making our CAR-T product candidates essentially 100% CAR-positive. This contrasts with competing products that do not utilize positive selection and typically contain a significant number of CAR-negative cells that cannot kill cancer cells but are artificially activated and expanded outside of the body and may contribute to CRS and/or neurotoxicity. Thus, we believe that positive selection is another mechanism, in addition to the high percentage of T_{SCM} cells, that may result in our CAR-T product candidates having a significantly greater therapeutic index.

Given that every CAR-T cell has a transgene, which is stably integrated into the genome, there is the possibility that the transgene delivery part of the CAR-T manufacturing process could create a detrimental mutation that allows the cell to expand in an uncontrolled manner, which can result in the cell itself becoming cancerous. Additionally, in the case of viral-manufacturing, some viral components that are integrated into the CAR-T cell as part of the transgene, such as the long terminal repeats, or LTRs, of the transgene may be able to activate a gene already in the cell, resulting in the cell becoming cancerous, a process called oncogenesis.

There has been an example of a clonal expansion in a patient who received a CAR-T product made from lentivirus. A clonal expansion means that a single T cell was given a proliferative advantage and was able to grow to a majority of all the CAR-positive cells in the patient. In this case, the clonal expansion was caused by the lentivirus inserting into a gene important for proliferation. Our CAR-T product candidates utilize our proprietary piggyBac technology. PiggyBac has shown low integration into intragenic regions, meaning that it is less likely to cause a detrimental mutation. Also, unlike retroviruses, piggyBac does not contain LTR sequences, but rather ITRs and other components which act as strong insulators, enhancing stable transgene expression and lowering risk of oncogenesis.

We have included a cellular safety switch in each of our product candidates as an additional safety mechanism. Both CRS and neurotoxicity are thought to be related to an overactive T cell response. Therefore, timely intervention to diminish the number of CAR-T cells should be an effective method of managing the majority of adverse events. We believe an ideal intervention technique is one that could be titrated such that not all CAR-T cells would be eliminated, leaving some for continued therapeutic effect.

Commercial Scalability

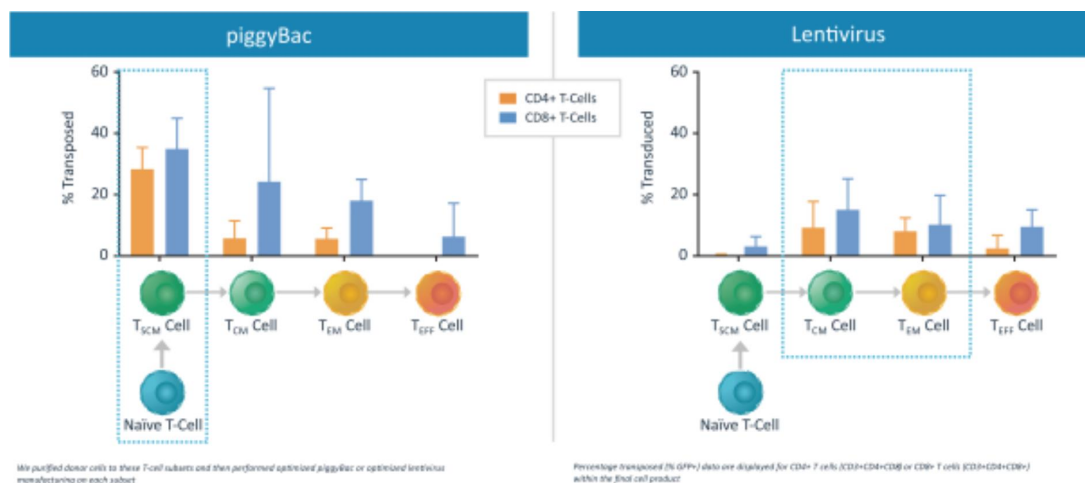
Another challenge with early-generation CAR-T products is their commercial scalability. Autologous CAR-T products are, by definition, individualized products. They are also typically expensive to produce, particularly when using viral-based manufacturing methods. We believe our non-viral piggyBac approach is more efficient and cost effective than historical CAR-T methods as it utilizes GMP nucleic acids, DNA and RNA, which are faster and cheaper to produce than GMP virus. We have further optimized the manufacturing process to eliminate some of the costly materials associated with the viral-based methods, including magnetic beads and cytokines.

CAR-T products that elicit severe and potentially fatal toxicities, such as CRS and neurotoxicity, require that the drug be administered in a tertiary care hospital where the physicians are familiar with treating these toxicities and where admission to an intensive care unit is an option. The potential for these severe toxicities currently precludes administration in community hospitals or outpatient infusion centers. In our dose-escalation Phase 1 clinical trial, as of November 16, 2020, to our knowledge no patient has had to be admitted to intensive care units for CRS or neurotoxicity. Based on these results, we initiated our potentially registrational Phase 2 clinical trial and, following discussions with the FDA, are dosing on a fully outpatient basis.

Efficacy Challenge: Elimination of CAR-T Cells

There are numerous explanations as to why CAR-T cells are eliminated from a patient after administration, but we believe the primary explanation is that the majority of T cells in other CAR-T products are more matured and short-lived T cells, including T_{EFF} cells. Not all T cells are created equally, and we believe the ability to develop a product that consists predominantly of early memory T cells, particularly T_{SCM} cells, is the key to increasing duration of response and tolerability. Our non-viral piggyBac manufacturing method is the only commercially viable approach known to us that can create CAR-T products with a high percentage of the highly desirable T_{SCM} cells with the efficiency of our technology.

In order to test the ability of our piggyBac DNA Delivery System to preferentially deliver CAR-containing transgenes to T_{SCM} cells, we conducted a preclinical experiment in which we separated T cells into their various subtypes, then individually put those subsets through either an optimized piggyBac manufacturing process or an optimized lentivirus process and measured the percentage of transposed or transduced cells in each final product subset. As shown in the figures below, piggyBac was very efficient at transposing (the piggyBac process of delivering the CAR-containing transgene) in T_{SCM} cells, while lentivirus was relatively ineffective at transducing (the lentiviral process of delivering the CAR-containing transgene) in T_{SCM} cells. We measured both CD4+ T cells (also known as T helper cells) and CD8+ T cells (also known as cytotoxic T cells) which represent two subsets of T cells believed to interact and be important in immune function and T cell response.

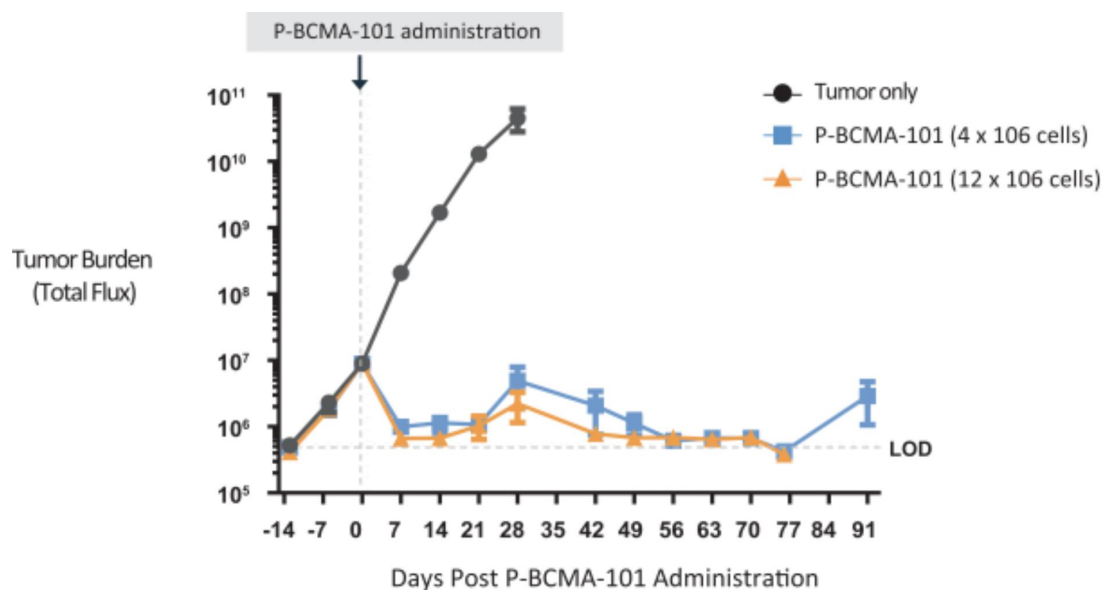


Given the one-way maturation pathway of T cells, we believe utilizing a genetic engineering method that preferentially modifies T_{SCM} cells is essential for creating a final product with a high percentage of T_{SCM} cells. During manufacturing, once we have completed the genetic modification step, we then perform a positive selection step to eliminate cells that have not been modified. Lastly, we activate and expand the remaining cells under conditions that favor self-renewal of T_{SCM} cells without differentiation, resulting in a product that has a high percentage of T_{SCM} cells, even when starting with patient material with a relatively low percentage of T_{SCM} cells. Our non-viral piggyBac DNA Delivery System typically yields T_{SCM} cell percentages reaching as high as 80%. We compared our piggyBac manufacturing method to a lentivirus-based manufacturing method that utilizes alternative media (Aim V, Thermo Fisher Scientific), different T cell stimulation (CD3/CD28 beads from Dynal/Thermo Fisher Scientific) and virus for vector integration (lentivirus). The sorted T cell subsets were put through the piggyBac process once in a pilot experiment with cells from one donor, and again in a comparison with the lentivirus process with cells from three donors. The early memory component, or combined T_{SCM} and T_{CM} cells, typically comprise greater than 90% of the cells of our product candidates. Notably, in December 2019, we were issued a U.S. patent that has claims that cover any modified T cell product that has 25% or more T_{SCM} cells.

Others in the field of CAR-T development are also attempting to increase the percentage of T_{SCM} cells in their products through alternative methods during the manufacturing process, including the addition of small molecule inhibitor drugs and various cytokines, reducing the time in culture, and physically enriching through sorting methods for early T cells. However, we believe these methods all have inherent problems that will limit the ability to successfully create a final product candidate with a high percentage of T_{SCM} cells.

In both our own clinical data and in data published and presented by others, a higher percentage of T_{SCM} cells in CAR-T products have been shown to correlate with clinical response, and our CAR-T product candidates contain a high percentage of T_{SCM} cells. Our goal is that our product candidates will overcome the limitations of other CAR-T products in many respects, including potency and durability of response.

The importance of these T_{SCM} cells can be seen in a preclinical model in which mice are implanted with a highly aggressive human multiple myeloma cell line (MM.1S). In this model, P-BCMA-101 engrafted with marked persistence *in vivo*, and remarkably, was able to control relapses without re-administration of product, as shown in the chart below:



We have also seen clinical evidence that our product candidates that are comprised of a high percentage of T_{SCM} cells can engraft and persist for exceptionally long periods in some patients. As of November 16, 2020, one patient from Cohort 2 of the Phase 1 clinical trial for P-BCMA-101 had been in a durable response for greater than 30 months. In another patient from Cohort 3 of the same clinical trial, we have recently observed our CAR-T modified cells in the peripheral blood at over 2 years post infusion. Because T_{EFF} cells are generally thought to live for a few weeks up to a few months, the presence of these cells in the patient’s blood at 2 years is evidence that some number of T_{SCM} cells have engrafted and continue to produce more differentiated cells to continue to fight the cancer. This patient was in a stringent complete response, or sCR, for over 2 years, as of November 16, 2020.

More matured T cells, which already have a short lifespan compared with T_{SCM} cells, can be eliminated from the patient due to their inability to persist, leading to poor efficacy of the product. One reason that premature loss of CAR-T occurs is the presence of CAR binding molecules on the surface of the T cell that can interact with each other. This results in crosslinking of the CAR molecule and a phenomenon called tonic signaling, in which the CAR-T cells are essentially always stimulated and active. Tonic signaling results in premature loss of efficacy, poor expansion and cell death, referred to as T cell exhaustion. We use binding molecules, such as Centyrins and heavy-chain-only antibody fragments, that are unable to crosslink and are resistant to tonic signaling.

Efficacy Challenge: Antigen Escape and Antibodies

Some CAR-T products have been shown to lose efficacy due to what is called antigen escape, which occurs when expression of a CAR-T target on a tumor cell is lost or drastically reduced due to selective pressure from the CAR-T therapeutic, resulting in an expansion of the tumor cells that have escaped the ability of the CAR-T to kill them. To avoid antigen escape, we have focused our efforts on selecting targets where we believe expression is less likely to be reduced. For example, BCMA is important for cell proliferation, and so is considered less likely to be lost by the tumor cell following CAR-T treatment. Likewise, PSMA plays a key role in modulating signaling pathways implicated in mCRPC and so may also be less susceptible to antigen escape.

Another method to prevent antigen escape involves pursuing multiple targets on the cancer cell with the same CAR-T product. The likelihood that a cancer cell will be able to simultaneously downregulate or lose expression of multiple targets, as opposed to any single target, is greatly reduced. While the genetic cargo capacity of viral vectors is quite limited, piggyBac has demonstrated the ability to deliver greater than 20 times more genetic cargo capacity, allowing transfer of multiple CAR molecule genes simultaneously. We believe the large genetic cargo capacity of piggyBac could allow us to further address antigen escape by including two or more CARs or TCRs on the same T cell. We have three Dual CAR programs currently in preclinical development designed to seek improved efficacy including potentially addressing antigen escape in some indications.

In our P-BCMA-101 Phase 1 clinical trial, we have observed that some patients have formed antibodies, also known as anti-drug antibodies in response to our treatment. This is not uncommon in biologic drug development, including CAR-T development. Based upon our data to date, it appears that anti-drug antibodies are more likely to form at higher dose cohorts. In our expanded Phase 1 clinical trial for P-BCMA-101 we are investigating additional dosing strategies that may reduce or eliminate the impact of anti-drug antibodies, including administering the dose in smaller cycles over the first 30 days and adding rituximab to the preconditioning regimen to potentially suppress any antibody response. Evaluation of these dosing strategies is ongoing in our expanded Phase 1 trial.

CAR-T in Solid Tumors

Efficacy Challenge

In addition to the standard concerns regarding persistence of T cells in the treatment of hematologic malignancies, there are factors that exacerbate this problem when using CAR-T products for the treatment of solid tumors. To date, the great majority of early-generation CAR-T products have not demonstrated significant responses in solid tumors and there are a number of potential explanations for this poor efficacy. First, it is possible that CAR-T cells have more difficulty accessing solid tumor cells. In some diseases, such as acute lymphoblastic leukemia, the tumor cells are easily accessible by the CAR-T cells. However, in most solid tumors, there are a number of factors that may make it more difficult for CAR-T cells to access the tumor. Second, it is possible that solid tumor cells have changes in expression of certain checkpoint genes that render them resistant to killing by T cells. Third, the center of many solid tumors is very hypoxic, or low in oxygen concentration, and this environment is not thought to be conducive to T cell function.

There have been a few exceptions to the poor efficacy of CAR-T in solid tumors, notably in glioblastoma multiforme and hepatocellular carcinoma, where treatment with CAR-T has led to complete responses, or a CR, in solid tumors. In these rare cases, the patient was treated with numerous administrations of CAR-T product. Though CAR-T cells are not as effective against solid tumor cells as they are against hematological tumor cells, this can potentially be overcome by giving multiple administrations of CAR-T, resulting in numerous waves of more matured T cells killing the cancer cells. This approach would be more viable if there were an unlimited number of cells with which to treat the patient. However, manufacturing early-generation CAR-T products is relatively time consuming and expensive, and the final product is comprised of a limited number of cells, thereby making this approach impractical for many patients.

All of our solid tumor product candidates, including P-PSMA-101, P-PSMA-ALLO1 and P-MUC1C-ALLO1, are comprised of a high percentage of T_{SCM} cells, which we believe are able to engraft, self-renew and mature into every T cell subtype, including the T_{EFF} cells, which can persistently attack the tumor until deep responses are potentially achieved. Therefore, we believe our CAR-T product candidates have the potential to achieve high rates of response against solid tumors with a single administration. P-PSMA-101 resulted in the elimination of tumor cells to undetectable levels in 100% of animals, with one incidence of a relapse in the low dose cohort, in a preclinical model of mCRPC in which immuno-deficient mice were implanted with solid tumors comprised of a human mCRPC cell line. To our knowledge based on published literature, no other product candidate or already approved cancer therapeutic has shown complete solid tumor elimination in any animal in this preclinical model.

Safety

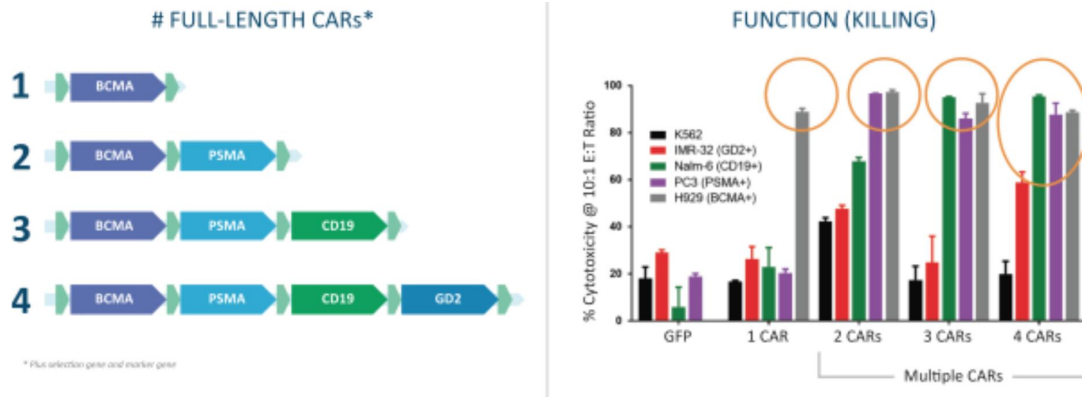
Our solutions for addressing CAR-T related toxicity concerns regarding CRS and neurotoxicity with respect to hematological tumors also apply to solid tumors. However, there are additional toxicity concerns for CAR-T

products when administered to treat solid tumors. When compared to hematological tumors, solid tumors generally have fewer unique surface targets that are not also expressed on healthy cells, so greater care must be taken when choosing targets to avoid on-target/off-tumor toxicity, which occurs when a CAR-T cell recognizes the intended target on a healthy cell and kills that cell. We seek to address this risk by choosing targets that are overexpressed in cancer cells, such as PSMA and MUC1-C, and by using binding molecules that we believe are more effective at binding the cancerous form of the target.

As we expand our solid tumor CAR-T pipeline, we expect it to become harder to identify targets that are unique to the solid tumor cells. Therefore, we are developing sophisticated systems designed to direct a CAR-T cell to kill a tumor cell based on presence or absence of a combination of targets. For example, we believe that we can develop a CAR-T that will kill only tumor cells that have both target A and target B on their surface but will not kill normal cells with target A or target B singularly on their surface.

A related strategy is developing a CAR-T that will kill a cell only if it expresses target A and B (which may be present on both cancer cells and normal cells) but not target C (which may only be present on normal cells). All such strategies require the co-expression of more than two CAR molecules on the surface of the same CAR-T cell. We believe the piggyBac DNA Delivery System can enable these approaches due to its large genetic cargo capacity. In contrast, viral-based approaches are typically unable to deliver more than two full-length CAR molecules.

We have demonstrated that we can produce CAR-T cells that express up to four full-length CAR molecule genes, each with a different target specificity, along with two additional genes, using a single piggyBac transposon in manufacturing (left panel). We further demonstrated that, when expressed, all CAR molecules perform specific killing of corresponding cell lines that express the target (right panel):



Specific killing was evaluated via reporter-based killing assays where the indicated human tumor cells were genetically modified to express the luciferase gene. These tumor cells were co-cultured in vitro with CAR-T cells for 24 hours at a defined effector to target ratio of ten to one (10:1). The CAR-T cells expressed different combinations of full-length CARs: (1) BCMA CARTyrin, (2) BCMA CARTyrin and PSMA CARTyrin, (3) BCMA CARTyrin, PSMA CARTyrin and CD19 scFv-based CAR or (4) BCMA CARTyrin, PSMA CARTyrin, CD19 scFV-based CAR and GD2 scFv-based CAR. Cytotoxicity (specific lysis) was evaluated by adding luciferin substrate and reading luminescence signal and percent cytotoxicity was calculated by enumerating the luminescence of tumor cells alone versus tumor cells with CAR-T cells. Each individual CAR demonstrated cytotoxicity against its cognate antigen, even when expressed in the presence of three additional full-length CARs.

Another approach to treating solid tumors is to express a variation of a TCR that is specific for a cancer-associated protein that is only expressed inside of the cancer cell, in contrast to a CAR molecule that only recognizes targets on the surface of the cell. We believe we can use the TCR strategy in combination with the CAR strategy by expressing combinations of both CAR and TCR molecules on the surface of the same cell using the piggyBac manufacturing method.

Commercial Scalability

We believe each of the commercial and scalability benefits of our approach in hematological tumors would also apply to solid tumors.

Allogeneic or Off-The-Shelf CAR-T Therapies

Efficacy Challenge

The goal of an allogeneic, or off-the-shelf, CAR-T product is to create a large number of doses of CAR-T from a single donor or cell line. A successful allogeneic CAR-T product could be used as an off-the-shelf product to treat any patient with a specific indication, thereby greatly decreasing the costs associated with manufacturing. However, if an allogeneic product requires high doses or multiple doses in order to achieve the same activity as a similar autologous product, then many of the potential cost-saving advantages of an allogeneic product would not be realized.

Gene editing tools are widely used to eliminate expression of certain cell surface molecules, which may be used to avoid the potential reactivity of donor cells against the patient, which results in graft-vs-host disease, or GvHD, as well as the reactivity of the patient's cells against the CAR-T product, a reaction called host-vs-graft. We believe it is imperative to use gene editing tools that can efficiently edit resting T cells when creating an allogeneic CAR-T product, as activating T cells will initiate the maturation pathway. Once T cells begin maturing, they start to lose their desirable T_{SCM} characteristics and thereby become exhausted, rendering the resulting product less efficacious.

Unlike many other gene editing technologies, Cas-CLOVER can efficiently edit resting T cells, allowing for the maintenance of the highly desirable T_{SCM} product composition in allogeneic product candidates, an important component of our CAR-T approach. Our goal with all of our allogeneic product candidates is to create a product with a profile comparable to or better than an autologous version of the same product; in the case of our first fully allogeneic product candidate for multiple myeloma, P-BCMA-ALLO1, our efficacy benchmark will be against P-BCMA-101 and other BCMA targeting programs.

Safety

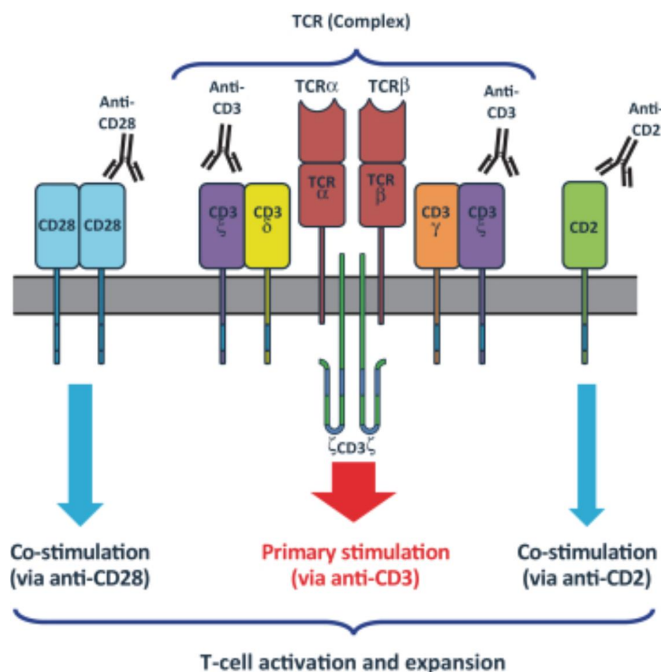
In addition to the standard concerns regarding CRS and neurotoxicity, there are additional safety concerns relative to an allogeneic product. As mentioned above, an allogeneic product can cause two forms of alloreactivity: GvHD and host-vs-graft. Host-vs-graft is concerning only in that it may cause premature elimination of the allogeneic CAR-T cells, resulting in all of the previously discussed efficacy challenges related to poor persistence of product, but it does not create a safety concern.

However, GvHD, a situation where the CAR-T cells are killing the healthy cells of the patient, is a serious and potentially fatal condition. Studies have suggested that the endogenous TCR is the molecule that needs to be eliminated in order to prevent GvHD. If this molecule is not completely eliminated in nearly 100% of CAR-T cells, then GvHD may become a problem. Our highly efficient Cas-CLOVER technology and subsequent purification step has resulted in cells that have TCR expression completely eliminated from at least 99% of the cells, a level we believe to be safely above that required to prevent GvHD.

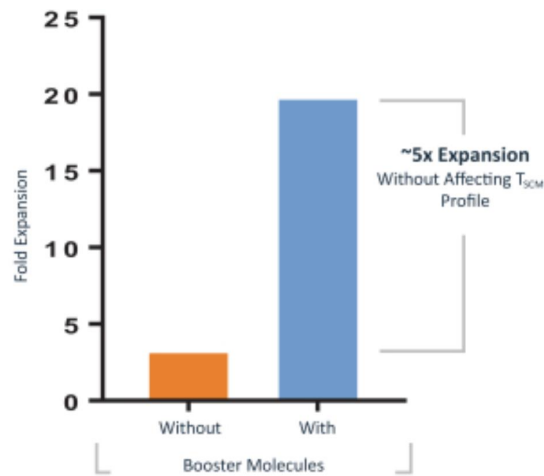
An advantage of an allogeneic product is that many doses can be generated from a single individual donor or cell line. However, a potential disadvantage is that any detrimental mutation created during manufacturing would be potentially present in doses given to many patients, as opposed to an autologous product where this risk is limited to the individual patient. Therefore, it is especially important to minimize or completely prevent unwanted off-target mutations. It is well known that some gene editing technologies, such as CRISPR, have the possibility of creating unwanted mutations. In preclinical testing, our Cas-CLOVER technology has shown precise site-specificity, having no or very little propensity for creating off-target mutations. Based on our own preclinical data and previously published results on other fully dimeric CRISPR systems, we believe Cas-CLOVER is the most specific gene editing method available.

A fully allogeneic CAR-T product would offer the possibility of significant time and cost savings in manufacturing, thereby greatly decreasing the cost per dose and increasing patient accessibility. Nonetheless, a manufacturing process must still be run on individual donor or cell line material in order to create a fixed number of doses of an allogeneic product. One of the most expensive parts of a manufacturing run for viral-based manufacturing methods is the virus itself. The piggyBac manufacturing system uses only GMP DNA and RNA without the need for GMP virus. We believe this will result in product candidates that are significantly cheaper to produce, even in the context of an allogeneic CAR-T product. Furthermore, the development and manufacturing timelines for piggyBac are shorter than those for virus, meaning one can move from product concept to GMP material more quickly. As an example, we moved P-BCMA-101 from product concept to the first patient dosed in a clinical trial in less than two years, and we believe we can apply these learnings to meet or exceed these timelines for future product candidates.

Genetic modification of the TCR, necessary to avoid GvHD as discussed previously, creates T cells that may be difficult to expand during the manufacturing process. TCR is commonly used as a key receptor for T cell stimulation in most autologous CAR-T manufacturing strategies. However, in allogeneic strategies, knockout of any single component of the TCR causes loss of the entire TCR complex from the surface of the engineered T cell, thereby significantly reducing its responsiveness to anti-CD3 antibodies during manufacturing. These consequences of eliminating the TCR and other genetic modifications have been commonly referred to as the “Allo Tax.” The TCR complex is depicted in the figure below.



We have developed proprietary booster molecules that have the potential to overcome this issue, while retaining and potentially increasing the percentage of T_{SCM} cells in the final product. Booster molecules are an RNA-based technology introduced to T cells during the manufacturing process, which results in transient expression of a receptor on the surface of T cells that allows the cells to respond to antibody-based activator molecules, resulting in significant expansion of the cells without causing maturation or exhaustion of the cells. The use of a proprietary booster molecule resulted in enhanced expansion and yield, resulting in the production of more than five-fold the number of cells than without the booster molecule from a single manufacturing run (see figure below).



We believe that we can create fully allogeneic product candidates, such as P-BCMA-ALLO1, P-PSMA-ALLO1 and P-MUC1C-ALLO1, that retain a profile that is comparable to their corresponding autologous products, as applicable, but with the ability to create enough doses to potentially treat hundreds of patients from a single manufacturing run.

Our CAR-T Product Candidate Pipeline

We believe we are particularly well-positioned to drive the continued advancement of CAR-T therapies in oncology. Our proprietary non-viral, gene engineering technologies are designed to address some of the greatest challenges to the successful implementation and commercialization of CAR-T therapies. We have built a wholly owned pipeline of autologous and allogeneic CAR-T product candidates, initially focused on the treatment of hematological malignancies and solid tumors.

P-BCMA-101: Autologous CAR-T for Multiple Myeloma

Overview

P-BCMA-101, our most advanced product candidate, is an autologous CAR-T therapy being developed for the treatment of patients with relapsed/refractory multiple myeloma. P-BCMA-101 targets BCMA, which is expressed on essentially all multiple myeloma cells. P-BCMA-101 utilizes several of our proprietary CAR technologies, including an anti-BCMA CAR molecule gene, a human DHFR gene, which is used to manufacture a highly purified product, as well as a safety switch gene, which we believe allows elimination of some or all of the P-BCMA-101 cells following treatment if desired by the clinician. All components of the P-BCMA-101 transgene are comprised of fully human sequences. We are currently enrolling in an expanded dose escalation Phase 1 clinical trial and plan to continue enrollment in a potentially registrational Phase 2 clinical trial in 2021.

The P-BCMA-101 CAR-T molecule utilizes an anti-BCMA Centyrin protein as the binding molecule, rather than an scFv antibody fragment used in most other CAR-T therapies. Centyrins, like antibody fragments, have high binding affinities and are target specific. Centyrins are also stable on the cell surface and do not form multimers, which we believe render them resistant to tonic signaling and T cell exhaustion.

P-BCMA-101 is engineered using our piggyBac DNA Delivery System. PiggyBac modification of human T cells requires only piggyBac transposon transgene DNA and RNA encoding piggyBac transposase, the enzyme that specifically mobilizes piggyBac transposon DNA, thereby eliminating the need for viral vectors and resulting in significant time and cost savings in manufacturing. P-BCMA-101 is produced with our proprietary manufacturing system that results in a highly purified product comprised of a high percentage of T_{SCM} cells, which we believe will drive more gradual tumor killing, thereby inducing less inflammatory cytokine response and improving the

tolerability profile of our CAR-T product candidates relative to those of existing CAR-T therapies manufactured using viral methods.

Target Indication

Multiple myeloma is a deadly form of blood cancer that develops from abnormal plasma cells, a type of immune cell that is typically responsible for secreting antibodies to fight infection. The underlying cause of multiple myeloma is unknown, but it affects patients by creating abnormal plasma cells that secrete high levels of antibodies, or fragments of antibodies, resulting in kidney and other organ malfunction that is ultimately fatal. It can also cause overproduction of abnormal plasma cells in the blood and tumor masses called plasmacytomas in the bone marrow or soft tissue.

There are approximately 100,000 patients suffering from multiple myeloma in the United States, with 30,000 new cases and nearly 13,000 deaths from the disease annually. It occurs more commonly in men than in women, typically affecting older adults, with the average age of onset of approximately 66 years. The current treatment paradigm in multiple myeloma begins with chemotherapy, proteasome inhibitors and immunomodulatory imide drugs, or IMiDs. The great majority of patients become refractory to these drugs and/or relapse, creating a high unmet need for treatments for relapsed/refractory patients. After failing proteasome inhibitors and IMiDs, patients typically resort to intensive chemotherapy regimens, with or without autologous stem cell transplant, or move to palliative care. Multiple myeloma is rarely cured, with the great majority of patients dying from the disease. Without treatment, the typical life span of a multiple myeloma patient is approximately seven months, while approximately half of those treated under the current regimens survive for five years after diagnosis. We believe P-BCMA-101, if successful in the clinic, can dramatically increase survival, as well as quality of life for relapsed/refractory multiple myeloma patients.

Clinical Data

The primary objectives of the Phase 1 clinical trial are to evaluate safety and any dose limiting toxicities, or DLTs, and determine the maximum tolerated dose, or MTD, of a single-dose infusion of P-BCMA-101 in adult patients with multiple myeloma who are relapsed and/or refractory to conventional therapy. In addition, we are assessing anti-myeloma response activity using the International Myeloma Working Group, or IMWG, criteria.

We initially focused on enrolling patients with relapsed/refractory multiple myeloma who have received at least three prior lines of therapy, including a proteasome inhibitor and an IMiD, and/or who are double refractory to a proteasome inhibitor and an IMiD. In 2019 we expanded the Phase 1 clinical trial to allow us to evaluate additional novel methods of treatment with CAR-T cells, including cyclic dosing or combinations with other agents. This expansion portion of the study is ongoing.

The original Phase 1 protocol allowed for enrollment of up to 40 adult subjects with multiple myeloma across five cohorts, using a standard 3 + 3 dose-escalation design. Eligible subjects who enroll in the study undergo leukapheresis to collect T cells for P-BCMA-101 manufacturing. Before administering the P-BCMA-101 product candidate, subjects received a standard conditioning lymphodepletion chemotherapy regimen of 300 mg/m² of cyclophosphamide and 30 mg/m² of fludarabine intravenously daily for three consecutive days, followed in two days by a single infusion of P-BCMA-101. Patients are then assessed for safety and efficacy for up to 15 years. Five additional exploratory cohorts were subsequently added to test the novel treatment regimens, allowing for enrollment of up to 80 additional patients for a total of up to 120 in the Phase 1 clinical trial. Each exploratory cohort allows for the same 3+3 dose escalation, but additionally assesses nanoplasmid manufactured P-BCMA-101, P-BCMA-101 given as 2-3 infusions each separated by 2 weeks, and/or administration of rituximab or lenalidomide before and after P-BCMA-101. Between late 2019 and early 2020, we transferred the P-BCMA-101 manufacturing process to a new site of our CMO, Lonza, and introduced process modifications designed to improve manufacturing performance. P-BCMA-101 product manufactured at this new facility and with the process modifications will be used for the remainder of Phase 1 expansion trial and the Phase 2 clinical trial.

We initiated the Phase 2 clinical trial in June 2019. Patients enrolled in the Phase 2 clinical trial will undergo the same management and assessments conducted during the Phase 1 clinical trial, with the intent of demonstrating a significant response rate and duration of response by IMWG criteria to support a biologics license application, or

BLA, submission for P-BCMA-101 for the treatment of multiple myeloma. We are seeking insights from our ongoing Phase 1 dose exploration study, which we expect to have in the first half of 2021 before fully engaging enrollment in the Phase 2 clinical trial using the methods determined to be optimal in the Phase 1 expansion cohorts. We expect that the Phase 2 clinical trial will enroll 120 patients.

P-BCMA-101 Phase 1 Clinical Trial: Interim Findings

As of the cutoff date of November 16th, 2020, 55 evaluable patients had been treated with no DLTs observed.

The median enrolled patient age was 60, and they had received up to 18 prior lines of therapy (median of 8), with all having received at least one proteasome inhibitor and at least one IMiD. 93% of these patients had received daratumumab and 67% of the patients had received an autologous stem cell transplant.

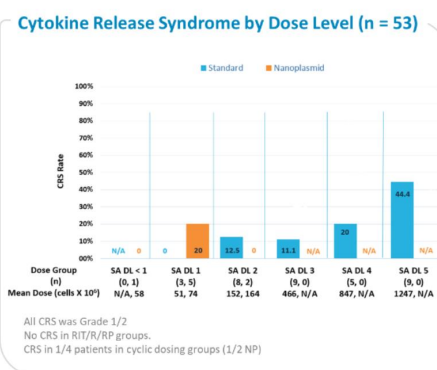
Interim Safety Results

Patients treated as of the November 16, 2020 data cutoff date and evaluable for safety results are summarized in the table below with the most common treatment-emergent adverse events, or TEAEs, and those considered particularly relevant to CAR-T cell products and lymphodepletion regimens. Grade 1 toxicities are generally considered mild, Grade 2 toxicities are moderate, Grade 3 toxicities are severe, Grade 4 toxicities are potentially life threatening and Grade 5 result in death.

No DLTs had been reported as of the data cutoff date. To our knowledge, no patient was admitted to an ICU for CRS or neurotoxicity, nor has the safety switch been used in any patient. Occasionally, cancer patients may be admitted to an ICU for reasons not directly related to treatment. Limited CRS has been reported with P-BCMA-101. As seen in the chart below, as of the November 16, 2020 data cutoff date, nine patients had cases of CRS (17%) reported. The rates of CRS trended towards being higher in the highest dose cohort although still lower than most comparator CAR-T cell products. One case of neurotoxicity (confusion) was observed in a patient with mental status changes prior to treatment, and another case (aphasia) was reported in a patient in conjunction with pneumonia and sepsis.

TEAE, n (%)	Overall (n=53)	≥ Grade 3
Dose Limiting Toxicity (DLT) ^a	0 (0.0)	0 (0.0)
Cytokine Release Syndrome ^a	9 (17.0)	0 (0.0)
Administered tocilizumab	4 (7.3)	
Administered steroids	3 (5.5)	
Neurotoxicity ^a	2 (3.8)	2 (3.8)
Infection		
Overall	24 (45.3)	10 (18.9)
First month	9 (17.0)	4 (7.5)
Neutropenia/Neutrophil count decreased ^b	41 (77.4)	40 (75.5)
Thrombocytopenia/Platelet count decreased ^b	22 (41.5)	16 (30.2)
Anemia	21 (39.6)	16 (30.2)
White Blood Cell Count Decreased	21 (39.6)	19 (35.8)
Fatigue	17 (32.1)	0 (0.0)

Highest incidence TEAE were infection, neutropenia, thrombocytopenia, anemia, leukopenia and fatigue
 No rimiducid use or ICU admission for CRS
 16 patients treated outpatient



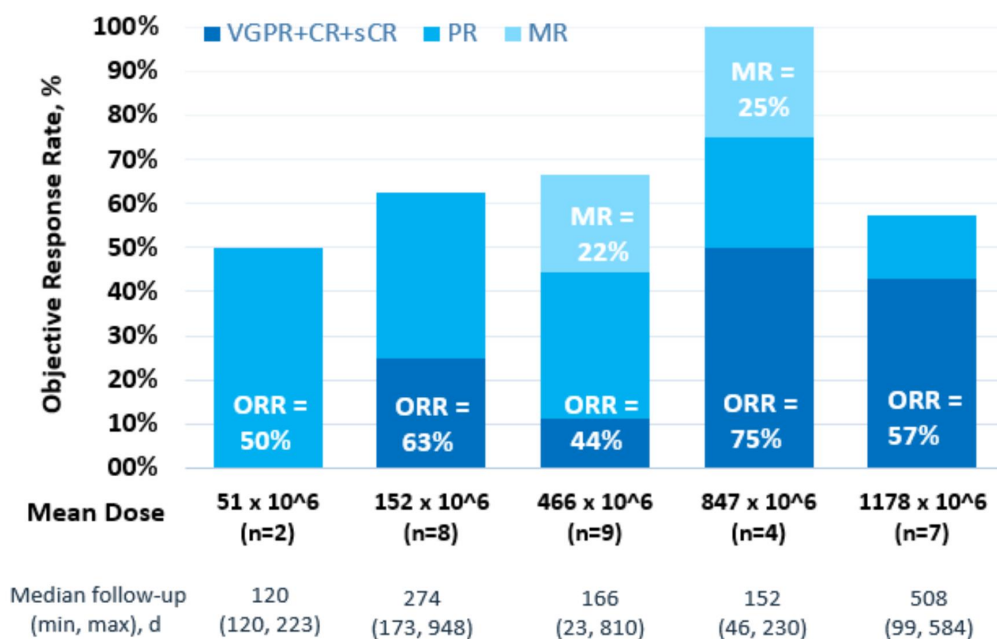
^aBy investigator assessment ^bPatient counted once for either term

Other than CRS, reported TEAEs and SAEs of interest included cytopenias, infections and constitutional symptoms which are consistent with conditioning lymphodepletion therapy and the underlying disease. No patient deaths have been reported as related to treatment with P-BCMA-101 as of the data cutoff date.

Interim Response Results

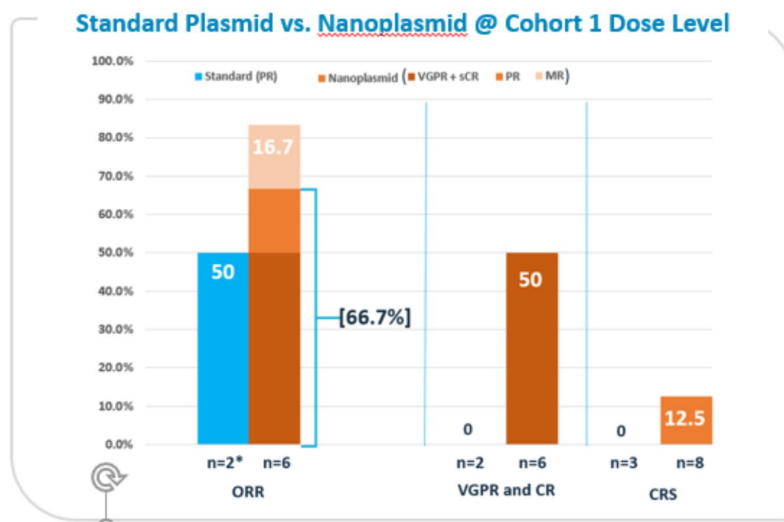
As of November 16, 2020, 43 patients treated with P-BCMA-101 were evaluable for response by International Myeloma Working Group, or IMWG, criteria. Of these patients, 23 (53.5%) responded with sCR, CR, VGPR or PR by IMWG criteria, and another 5 (11.6%) with MR. Other patients were not evaluable by IMWG criteria due to insufficient myeloma protein markers at the time of treatment or did not yet have a response assessment available at the cutoff date.

The following chart presents the best objective response rate, or ORR, for IMWG evaluable patients by dose group, with a breakdown by depth of best response during the initial dose escalation:



Data cutoff November 16, 2020

An additional 11 patients treated in exploratory cohorts with the modified nanoplasmid based manufacturing process were evaluable by the cutoff date. Data from evaluable patients in this group matched by dose level to patients in the initial escalation using the original manufacturing process are presented in the table below. Overall, the response rate appears to be improving with this manufacturing process, while the product candidate has remained generally safe and well-tolerated.



Under the original Phase 1 protocol, we had provided for the testing of minimal residual disease, or MRD, only after patients reached a CR. In the time since the original Phase 1 protocol was established, MRD has become better understood to correlate with CRs and the risk for relapse. As a result, we modified the protocol to test patient MRD status earlier in the process and that testing is underway. Based on a limited number of samples as of December 3, 2020, 6 of 29 evaluable Phase 1 patients had at least one sample test negative for MRD.

Given the tolerability observed in our trial, we expanded the current Phase 1 clinical trial to explore additional dosing regimens, including administering the product over several cycles during the first month after lymphodepletion conditioning and adding rituximab to the preconditioning regimen, which we believe may further improve response rates, depth of response and/or durability of response.

Future Clinical Development Strategy

Given the clinical results generated to date, we plan to complete additional dose exploration in the Phase 1 trial before we accelerate enrollment in the Phase 2 clinical trial. The Phase 2 clinical trial will evaluate response rates and duration of response, the same endpoints used for a number of approved multiple myeloma therapies, such as daratumumab, bortezomib and carfilzomib, in an effort to support a BLA submission. Subsequently, we plan to conduct additional Phase 2 and comparative Phase 3 clinical trials to support approval, if required, and label expansion into earlier lines of monotherapy and combination therapies. Should data from our P-BCMA-ALLO1 program, which are anticipated to begin to be available in late 2021, meet our expectations, we would consider reallocating our efforts and resources to advancing our allogeneic program. P-BCMA-101 was granted FDA Regenerative Medicine Advanced therapy Designation in November 2018 and Orphan Drug Designation in May 2019.

P-BCMA-ALLO1: Allogeneic CAR-T in Multiple Myeloma

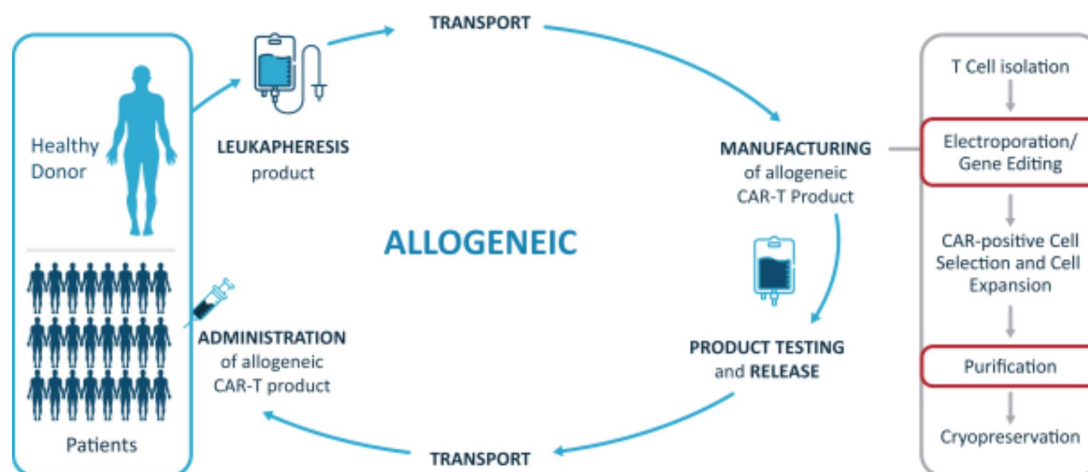
Overview

P-BCMA-ALLO1 is a fully allogeneic CAR-T product candidate being developed to treat multiple myeloma. P-BCMA-ALLO1 is in late preclinical development and we anticipate an IND filing and initiation of a Phase 1 clinical trial for P-BCMA-ALLO1 by the first half of 2021.

P-BCMA-ALLO1 is our first fully allogeneic CAR-T product candidate derived from healthy donor cells, giving it the potential to be used as an off-the-shelf therapy for unrelated multiple myeloma patients. We believe our technology and manufacturing processes are ideally suited to develop allogeneic CAR-T product candidates with

reduced alloreactivity and without unwanted mutations. We use our proprietary Cas-CLOVER gene editing tool to genetically engineer T cells in order to reduce or eliminate both GvHD and host-vs-graft alloreactivity. Cas-CLOVER is designed to efficiently edit resting T cells and has demonstrated precise specificity, thereby limiting unwanted off-target mutations and helping to improve tolerability.

The manufacturing process for P-BCMA-ALLO1 shares characteristics with P-BCMA-101, differentiated only by the process of a multiplexed gene editing step and a purification step. Both product candidates include a DHFR gene used to manufacture a highly purified product that is essentially 100% CAR-positive.

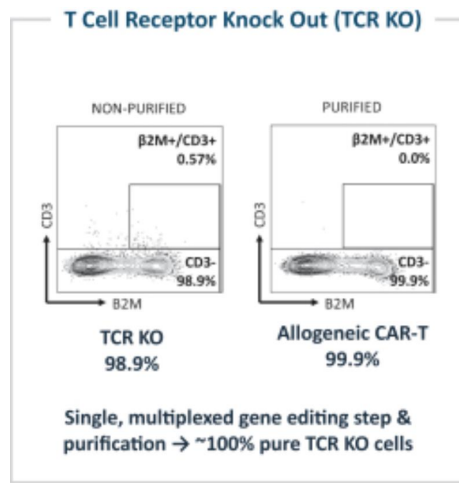


We believe an allogeneic product with a profile similar to an autologous product would have significant advantages in terms of cost and commercial reach, with the ability to treat potentially hundreds of patients from a single manufacturing run.

Preclinical Data

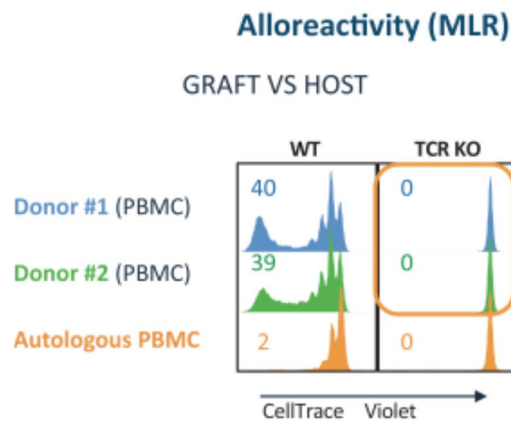
In our preclinical studies for our allogeneic product candidates we undertake gene editing to evaluate our ability to address both graft-vs-host and host-vs-graft reactions. We used our proprietary Cas-CLOVER gene editing platform, which has the ability to multiplex and efficiently edit resting T cells, to eliminate expression of cell surface proteins that are responsible for alloreactivity in a single gene editing step, followed by a purification step.

The figure below demonstrates our highly efficient gene editing to disrupt the T cell receptor β chain (TCR β) gene. In the experiment represented below, complete elimination of all TCR expression occurred in over 90% of cells with a single gene editing step and, after a single purification step, we were able to achieve a product candidate with >99.9% of cells with a TCR knockout. For P-BCMA-ALLO1, we also address host-vs-graft alloreactivity in the same multiplexed gene editing step by disrupting the Beta-2 Microglobulin gene (MHC1). With a single gene editing step we typically eliminate approximately 50-60% of MHC1. For P-BCMA-ALLO1 we do not purify for MHC1 knockout so that the final product is on average approximately 60% MHC1 knockout and >99.9% TCR knockout.



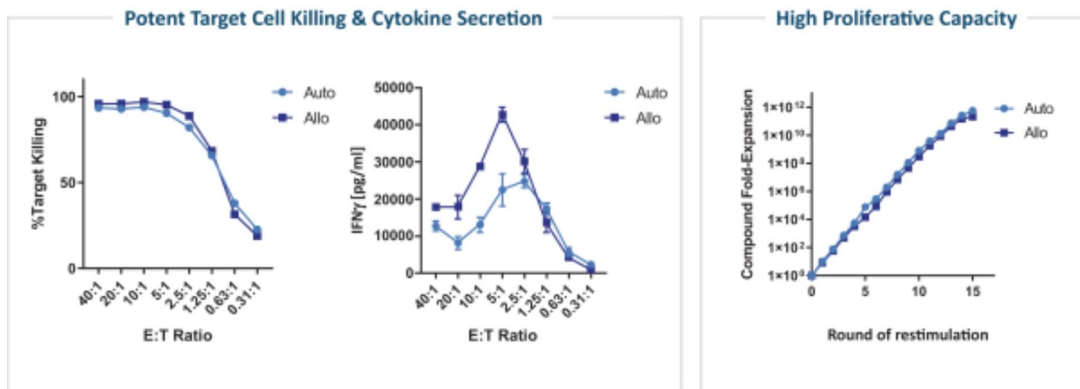
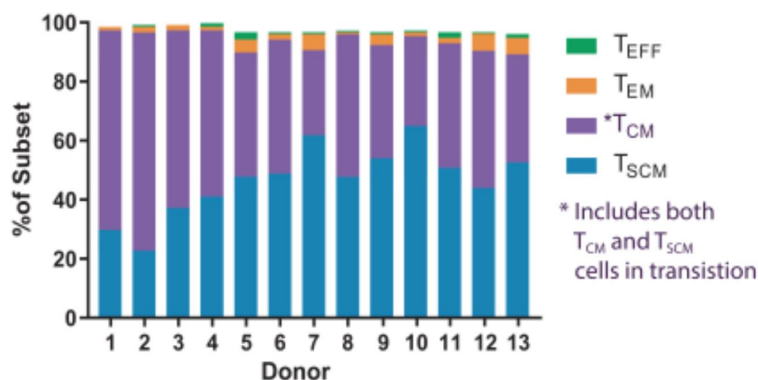
*Data presented at ASH 2017

Multiple preclinical experiments demonstrate the potential of P-BCMA-ALLO1 to reduce or eliminate alloreactivity. The below figure represents an experiment for graft-vs-host alloreactivity, which is normally mediated by the intact TCR. The panel shows results of a mixed lymphocyte reaction, or MLR, where alloreactivity was demonstrated by a peak forming on the left-hand side of the graph. Peaks were clearly seen when non-genetically modified cells, or wild type, or WT, were mixed with cells from an unrelated donor, but not when mixed with cells from the same donor. Alloreactivity was eliminated when testing the P-BCMA-ALLO1 cells.



The cells used in MLR assays were WT cells or TCR KO cells, and peripheral blood mononuclear cells, or PBMC, from either the same donor (Autologous PBMC) or PBMC from allogeneic donors (Donor #1 and Donor #2). These cells were labeled with one of two cytosolic dyes: CellTrace Violet for the WT or TCR KO cells. A portion of each labeled cell type was irradiated (3,000 rads) to arrest proliferation and possibly increase immunogenicity. GvHD reactions were modeled by co-culturing non-irradiated WT or TCR KO cells with irradiated PBMC. In the 12-day proliferation MLR assay, the response of the non-irradiated cell type was assessed by flow cytometry and visualized as histograms in which the undivided cells retain high levels of cytosolic dye and thus appear on the right-hand side of the histogram and those cells that have undergone a proliferative response will dilute the cytosolic dye with each division, thereby appearing in peaks shifted to the left. Cells that have divided more than six times are beyond the sensitivity of this experiment and accumulate in one peak on the far left of the histogram. The frequency (average, n=4) of the cells with fully diluted cytosolic dye appears above the histogram in each panel.

One of our goals for our P-BCMA-ALLO1 product candidate is to preserve the same high percentage of T_{SCM} cells in the final product that we have observed with our P-BCMA-101 and solid tumor autologous product candidates. Our first 13 research-scale manufacturing runs for P-BCMA-ALLO1 resulted in high levels of T_{SCM} as shown in the figure below.



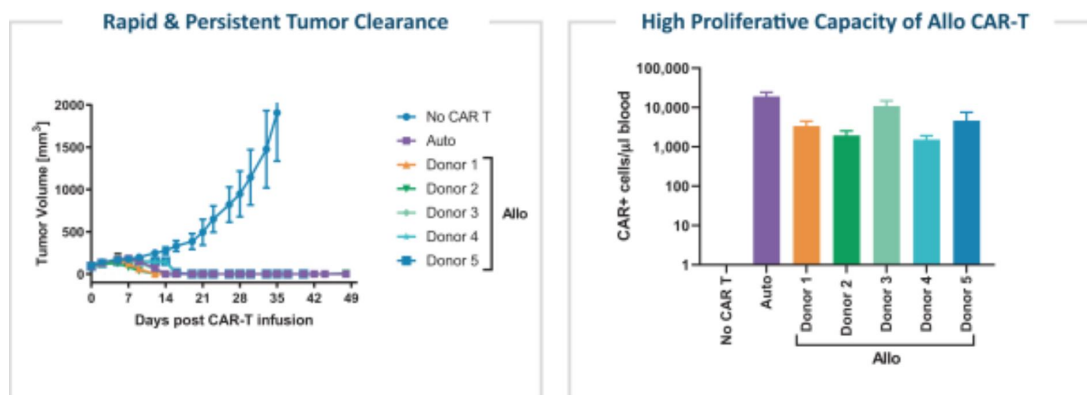
Further, we have demonstrated that in preclinical models P-BCMA-ALLO1 had comparable intensity and specificity of killing target cells as an autologous version, equal or better cytokine secretion, as well as equivalent proliferative capacity.

Cytotoxicity was measured in a standard *in vitro* killing assay. Primary healthy donor human T cells were taken through one of two production processes: Autologous, or Auto, cells were generated using the P-BCMA-101 production process and allogeneic, or Allo, cells were generated using the P-BCMA-ALLO1 manufacturing process. The two T cell products were mixed at effector to target ratios shown with target cells that were BCMA-positive. In this assay, both target cell lines also express the luciferase protein and the amount of live and intact target cells in culture can be indirectly assessed as a function of the luciferase enzyme that they contain. After 48 hours of co-incubation, cytotoxic activity by the T cell products is evident as a decrease in the amount of live and intact target cells and expressed as data normalized to the same value for target cells cultured alone (i.e., 0% cytotoxicity is seen without T cells). The data above demonstrated that Auto and Allo were specific in killing the BCMA-positive cell lines. Moreover, the specific killing increased with higher effector to target ratios and the fact that both Auto and Allo products demonstrated virtually identical trends at each effector to target ratio indicates that both products have similar intensity and specificity of killing.

In the panel on the right in the figure above, proliferative capability of both Auto and Allo were assessed in an *in vitro* serial restimulation assay. Auto (CD3+) or Allo (CD3-) CAR-T cells, as described above, were thawed and then stimulated every 4-5 days with irradiated RPMI-8226 tumor cells which express the CAR target antigen, BCMA. At each stimulation, CAR-T cells were counted and reseeded with target cells at a 2:1 effector to target

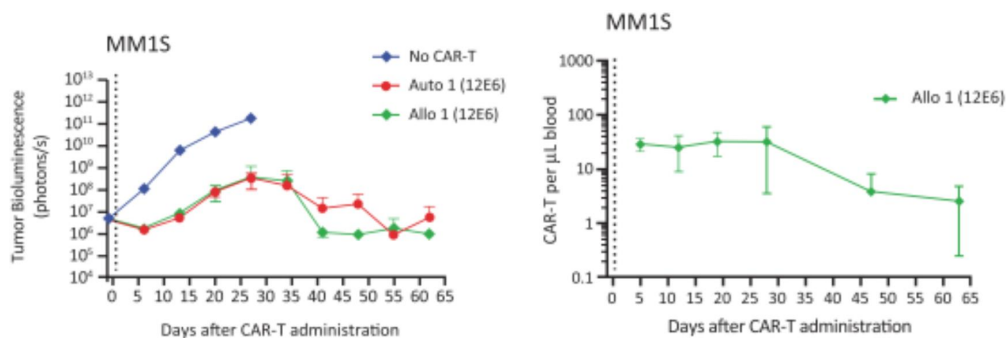
ratio. This assay demonstrates that CAR-T cells, both autologous and allogeneic, made with piggyBac have a high proliferative capacity and are able to expand to high cell numbers in response to tumor antigen. Importantly, it also shows that loss of the TCR complex in our P-BCMA-ALLO1 production process does not negatively affect the ability of our CAR-T cells to proliferate in response to antigen as our allogeneic cells could expand to a similar extent and for a similar length of time as CD3-replete autologous CAR-T product cells.

We also have demonstrated that P-BCMA-ALLO1 performs equal to or better than an autologous BCMA product generated from a healthy donor in an *in vivo* mouse model. As noted in the left panel of the figure below, we took five random healthy donors to generate P-BCMA-ALLO1 (Donor 1 to Donor 5) and compared those allogeneic products to an autologous BCMA CAR-T product produced from a healthy donor. As shown, all of the allogeneic products performed at least as well as the autologous product. In the panel on the right, we also compare the proliferative capacity of P-BCMA-ALLO1 to an autologous BCMA CAR-T product produced from a healthy donor.

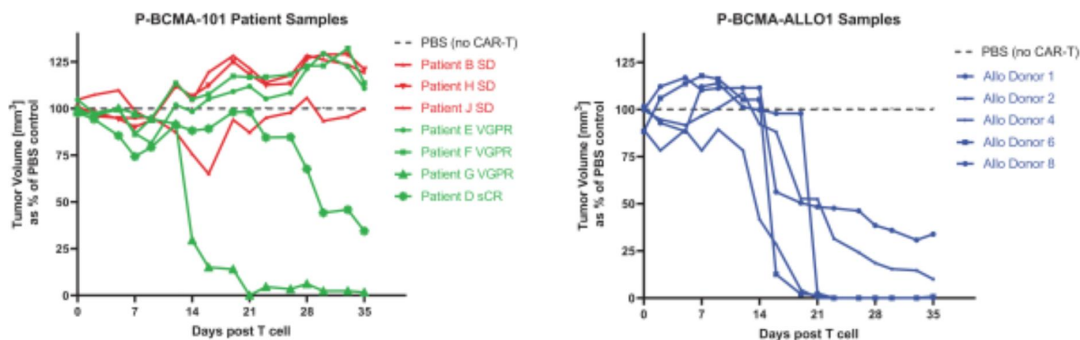


P-BCMA-ALLO1 exhibited potent anti-tumor effects in an *in vivo* mouse model of multiple myeloma. P-BCMA-ALLO1 was tested in alongside with healthy donor non-edited cells (Auto, CAR-positive/TCR-positive) in an *in vivo* model of multiple myeloma tumor control. Immunocompromised mice were implanted subcutaneously with 1×10^7 RPMI-8226 BCMA+ tumor cells and tumors were established for 7 days before injection with 1×10^6 CAR-T cells. P-BCMA-ALLO1 exhibited potent and sustained anti-tumor efficacy that was comparable to non-edited control CAR-T cells. P-BCMA-ALLO1 also demonstrated robust *in vivo* proliferation that could be detected in the blood of treated animals by Day 14 after T cell administration. The peak of expansion (Day 14) correlated with the timing of tumor control observed and was similar to expansion levels observed for TCR-positive autologous CAR-T cells.

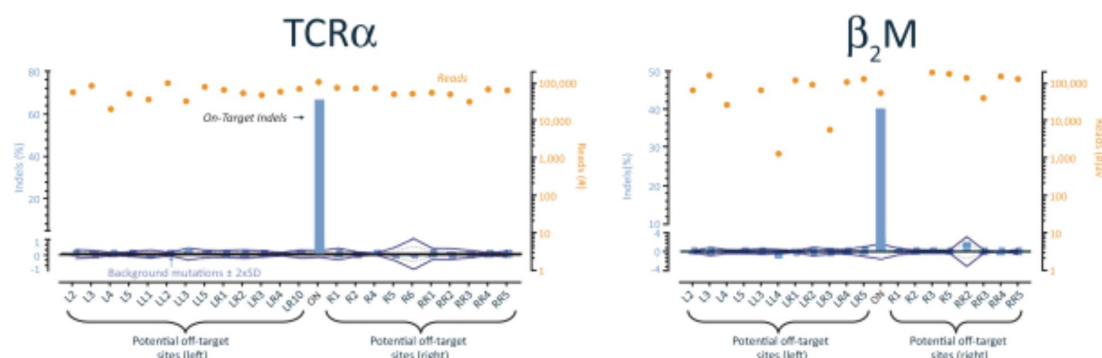
P-BCMA-ALLO1 also exhibited potent anti-tumor effects in an *in vivo* mouse model of multiple myeloma known as the MM1S model shown in the figure below. MM1S is an aggressive model of relapsed/refractory multiple myeloma where relapses after initial control of the tumor can be observed. Immunocompromised (NSG) mice were implanted intravenously with 3×10^5 MM1S tumor cells and tumors were established for 7 days before injection with a standard dose of 12×10^6 CAR-T cells. P-BCMA-ALLO1 (Allo) exhibited potent and sustained anti-tumor efficacy that was comparable to non-edited control autologous CAR-T cells (Auto) at the same single dose. P-BCMA-ALLO1 also showed persistence in this model as cells could be detected in the blood of treated animals at greater than 60 days post-CAR-T cell administration.



In addition, we have demonstrated that P-BCMA-ALLO1 performs equal to or better than autologous P-BCMA-101 products generated from cancer patients in an *in vivo* mouse model with a very high positive predictive value for how a product candidate will perform in the clinic. In the same mouse model (figure below), we used five random healthy donors to create P-BCMA-ALLO1 and compared those allogeneic products to seven P-BCMA-101 patient products, representative of patients that responded well (green; VGPR or sCR) or did not respond (red; SD) in the clinic. As shown, all the allogeneic products performed at least as well as the P-BCMA-101 products that resulted in either a VGPR or sCR in the clinic. Two of the four P-BCMA-101 products that demonstrated favorable outcomes in the clinic were able to control tumor in the mouse model. None of the three P-BCMA-101 patient products that resulted in SD in the clinic were able to control tumor in the mouse model.



Key to the creation of an allogeneic product is the specificity of the gene editing, without causing unwanted cuts or mutations in the DNA. Importantly, we demonstrated that Cas-CLOVER exhibited a high degree of specificity for on-target cutting during the cutting of gene targets in the production of P-BCMA-ALLO1. We performed deep sequencing of numerous top-ranked predicted off-target sites corresponding to these gene targets, and we have not observed any evidence of off-target activity:

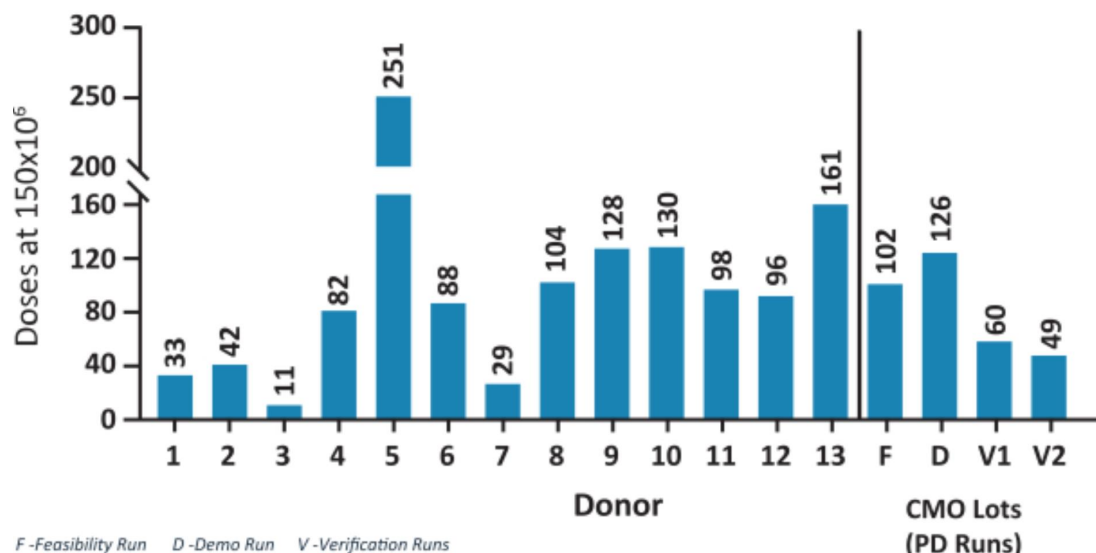


Cas-CLOVER can be used to efficiently knock-out several human T cell surface marker genes, such as TCR β and β -2 Microglobulin (β 2M). To determine the level of off-target activity by Cas-CLOVER, next generation sequencing was used to investigate T cells gene-edited for TCR β or β 2M genes. To do so, an algorithm was designed to predict all potential off-target sites of high DNA homology throughout the whole human genome for Cas-CLOVER gRNA. As Cas-CLOVER functions as an obligate homodimer, there are several hypothetical possibilities of dimer formation including left (L) gRNA + right (R) gRNA heterodimer, L + L and R + R homodimers, as well as L only and R only. On-target site and the top predicted off-target site amplicons from the above five different hypothetical combinations were PCR-amplified and the amplicons were analyzed using the Illumina Mi-Seq platform for deep sequencing with approximately 30,000 to 100,000 coverage at each locus for the identification of insertions and/or deletion, or indels.

In the above figure, the right Y axis shows the deep sequencing coverage rate at each locus, whereas the left Y axis shows the percentage of indels. The dotted lines show the level of background mutation in the absence of gene editing while the solid line shows the 2 standard deviation error bar of the control. The bars show the percentage of indels of all on- and off-target sites. These data show that the indels resulting at predicted potential off-target sites of high DNA homology are within the range of the background mutation rate of the non-edited negative control. Thus, next generation sequencing data confirmed that gene editing only occurs at target sites. Next generation sequencing data further showed that there is no off-target editing among the top predicted off-target sites.

We have developed proprietary booster molecules, which are an RNA-based technology introduced to T cells during the manufacturing process, results in transient expression of a receptor on the surface of T cells that is designed to allow the cells to respond to antibody-based activator molecules, resulting in significant expansion of the cells without causing maturation or exhaustion of the cells. Using booster molecules, we believe we can expand P-BCMA-ALLO1 cells to large numbers without losing any of the desirable cell attributes shown previously. In a preclinical study, we measured cell expansion of allogeneic CAR-T cells with and without the use of a booster molecule, and observed an approximately five times greater expansion during a single manufacturing run with the booster molecule, when compared to a manufacturing run without using a booster molecule. We continue to improve the booster molecule technology and other allogeneic manufacturing technologies and believe we will be able to significantly increase the number of doses generated per allogeneic manufacturing run in the future.

We estimate that we can generate enough cells from a single manufacturing run to treat dozens to hundreds of patients. Data from our first 13 random healthy donors selected only for minimal viral testing and put through our near clinical-scale manufacturing process and the first full-scale clinical manufacturing process at our CMO resulted in between 11 and 251 doses of 150M cells per dose, as shown below. We will continue to further optimize manufacturing and to identify donor characteristics that could be predictive of better performance.



Clinical Development Strategy

We anticipate an IND filing and initiation of a Phase 1 clinical trial for P-BCMA-ALLO1 in the first half of 2021. The trial will be an open-label dose escalation trial enrolling up to 40 patients.

P-PSMA-101: Autologous CAR-T for metastatic castrate resistant prostate cancer

Overview

P-PSMA-101 is a solid tumor autologous CAR-T product candidate being developed to treat mCRPC. P-PSMA-101 targets cells that express PSMA, which is highly expressed on mCRPC cells. PSMA is involved in folate uptake and is thought to confer a proliferative advantage to PSMA-expressing tumor cells. Additionally, PSMA levels increase as tumor cells become androgen-independent, a hallmark of advancing prostate disease. Therefore, we believe that PSMA may be less susceptible to antigen escape. P-PSMA-101 is currently being evaluated in a Phase 1 clinical trial.

The piggyBac transposon transgene of the P-PSMA-101 product candidate differs from P-BCMA-101 only in the binding (Centyrin) portion of the CAR molecule used, which we believe helps to reduce development and manufacturing risk by leveraging the experience gained with P-BCMA-101. As with P-BCMA-101, P-PSMA-101 includes a DHFR gene used to manufacture a highly purified product. Also, as with P-BCMA-101, P-PSMA-101 is produced with our proprietary manufacturing system that results in a highly purified product with a cell composition comprised of a high percentage of T_{SCM} cells, with the goal of conveying numerous benefits over other CAR-T products manufactured using viral methods.

Target Indication

Prostate cancer is the fourth most common cancer globally and the second leading cause of cancer death among men in the United States, with a 60% occurrence rate in men over the age of 65. In the United States alone,

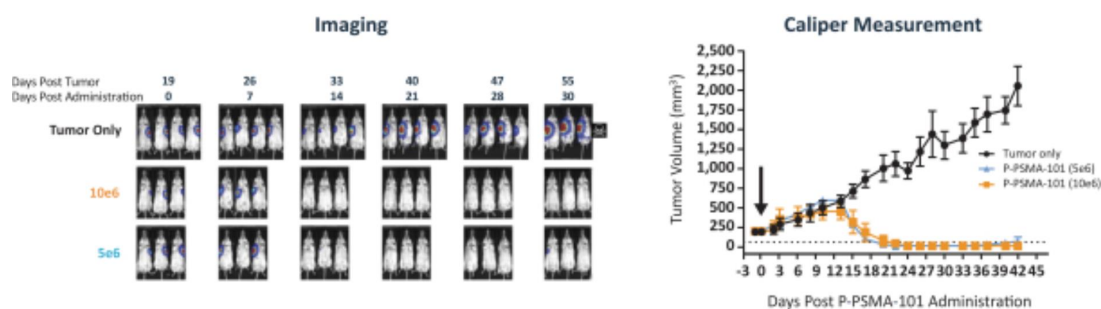
there are approximately 2.8 million men living with prostate cancer, with approximately 40,000 new cases of mCRPC estimated each year. The majority of prostate cancer patient deaths in the United States are due to mCRPC.

Treatment paradigms for prostate cancer vary based on the age of the patient at the time of diagnosis. Typical early treatment options for prostate cancer range from active surveillance, radiation therapy, cryotherapy, immunotherapy, hormone therapy and surgical treatment. For metastatic disease, the paradigm bifurcates between hormone naïve disease and castrate resistant prostate cancer, or CRPC. CRPC cases are typically treated with the chemotherapy drug docetaxel, and a choice of abiraterone, enzalutamide, cabazitaxel and/or Radium-223. Typically, none of these therapies are curative.

Although five-year survival rates for patients with early prostate cancer are nearly 100%, a high unmet need for mCRPC remains, with a five-year survival rate of only approximately 30%. We believe P-PSMA-101, if successful in the clinic and approved, could dramatically increase survival, as well as quality of life for mCRPC patients.

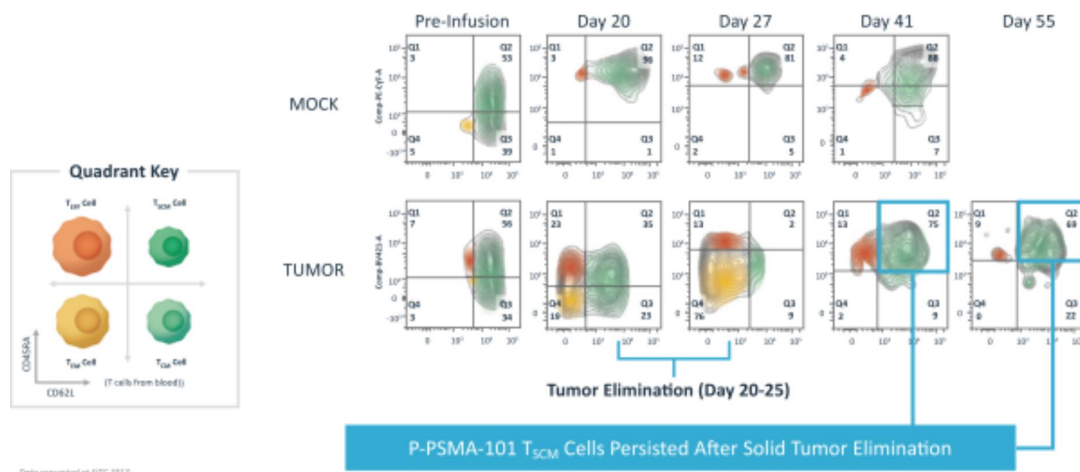
Preclinical Data

P-PSMA-101 resulted in the elimination of tumor cells to undetectable levels in 100% of animals, with one incidence of a relapse in the low dose cohort, in a preclinical model of mCRPC. This preclinical model involves the implantation of subcutaneous solid tumors comprised of a human mCRPC cell line (LNCaP (fLuc+)) in immuno-deficient mice. These tumors were well established to a size of at least 100 mm³ before administration of P-PSMA-101. In the model shown below, we demonstrated elimination of tumors to below the limit of detection by both bioluminescence imaging measurements (left side of figure) or caliper measurements (right side of figure) in 100% of animals with both a standard dose of 10 million P-PSMA-101 cells per animal (10×10^6), as well as a low dose of five million cells per animal (5×10^6). To our knowledge based on published literature, no other product candidate has shown complete solid tumor elimination in this preclinical model:



P-PSMA-101, comprised of a high percentage of T_{SCM} cells, expanded *in vivo* and gave rise to CAR-positive T cells that were more matured, including T_{EFF} cells, which were detected in the peripheral blood at early timepoints, followed by a decrease in tumor burden to below detectable levels as measured by both bioluminescent imaging and caliper.

Consistent with our hypothesis, the short-lived, more matured T cells were then eliminated, and the long-lived T_{SCM} cells engrafted and persisted and were the only cells detectible in the peripheral blood at later timepoints. Thus, even after solid tumor elimination, a population of P-PSMA-101 T_{SCM} cells persisted. The figures below show that in mice with no tumor, T_{SCM} cells engrafted and persisted without *in vivo* expansion and differentiation. In contrast, T_{SCM} cells expanded and differentiated in the presence of tumor in subject mice treated with P-PSMA-101, and continued to persist following solid tumor elimination:



Clinical Development Strategy

We filed an IND in late 2019 and received authorization to proceed to clinical trials in early 2020. We dosed our first patient in May 2020 and resumed enrollment in November 2020 following the clinical hold on the program from August 2020 to November 2020. Enrollment continued after approval of a protocol amendment intended to increase patient compliance and safety that includes modified inclusion and exclusion criteria and frequency of monitoring and laboratory testing. The current Phase 1 protocol allows for enrollment of up to 40 adult subjects with mCRPC across four arms of up to five dose escalation cohorts each, using a standard 3 + 3 dose-escalation design. Eligible subjects who enroll in the trial undergo leukapheresis to collect T cells for P-PSMA-101 manufacturing. Before administering the P-PSMA-101 product candidate, subjects will receive a conditioning lymphodepletion chemotherapy regimen. In the first arm, the regimen will be 300 mg/m² of cyclophosphamide and 30 mg/m² of fludarabine intravenously daily for three consecutive days, followed in two days by a single infusion of P-PSMA-101. In a second arm, rituximab will be added to the lymphodepletion regimen. The remaining two arms will utilize these same lymphodepletion regimens, but multiple infusions of P-PSMA-101 will be administered in two-week intervals, and the lymphodepletion regimen may be repeated every six weeks twice more. Patients will then be assessed for safety and efficacy for up to 15 years. Part 2 of the trial will allow expansion of selected cohorts to further characterize outcomes for a potential recommended Phase 2 dose. A Phase 2 pivotal part or additional exploratory cohorts may be added to the trial depending on the initial findings.

P-PSMA-ALLO1

P-PSMA-ALLO1 is an allogeneic CAR-T product candidate targeting PSMA and being developed to treat patients with mCRPC. We have designed P-PSMA-ALLO1 to be fully allogeneic, with genetic edits to eliminate or reduce both host-vs-graft and graft-vs-host potential reactions. We anticipate an IND filing and initiation of a Phase 1 clinical trial after analyzing preliminary results observed in the ongoing P-PSMA-101 Phase 1 clinical trial.

P-MUC1C-ALLO1: Allogeneic CAR-T in Multiple Solid Tumor Indications

Overview

P-MUC1C-ALLO1 is a preclinical allogeneic CAR-T product candidate with the potential to treat a wide range of solid tumor indications. The target, MUC1-C, is a tumor selective, aberrantly glycosylated, cleavage product of MUC1, that is highly expressed on most epithelial tumors.

We used our proprietary piggyBac DNA Delivery System to manufacture a highly purified P-MUC1C-ALLO1 product candidate containing a high percentage of T_{SCM} cells that we believe may be the key to developing a CAR-T therapy to treat solid tumors. We use our proprietary Cas-CLOVER platform to genetically engineer T cells in order to reduce or eliminate both GvHD and host versus graft alloreactivity.

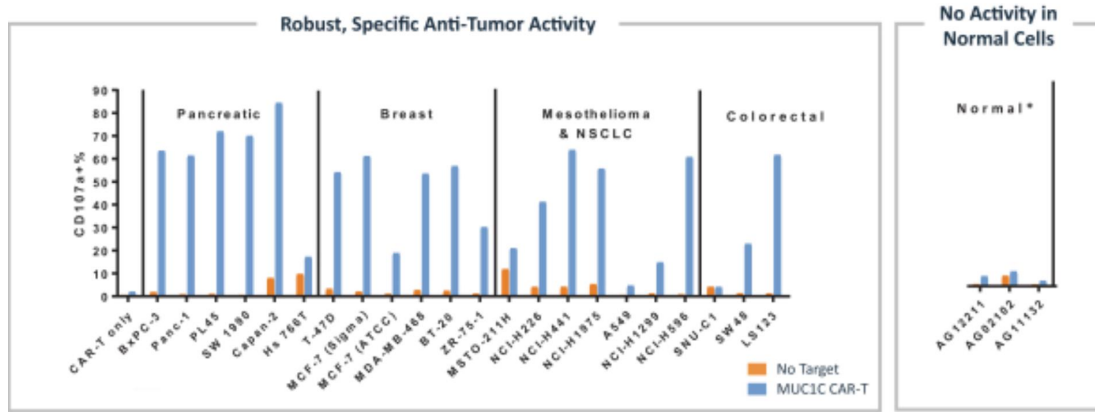
P-MUC1C-ALLO1 is currently undergoing preclinical development and we anticipate an IND filing and initiation of a Phase 1 clinical trial by the end of 2021.

Target Indication

We intend to further evaluate and later determine clinical indications for initial development of P-MUC1C-ALLO1 in indications where MUC1-C expression occurs. Approximately 90% of cancers derive from epithelial tissues, and among these cancers a significant percentage express MUC1-C, including common cancers such as breast, colorectal, lung, ovarian, pancreatic, renal and other cancers.

Tumor Type	MUC1 Expression (%)
Breast	91
Colorectal	81
Esophageal	32
Gastric	77
H&N SCCa	82
Mesothelioma	75
Multiple myeloma	59
Nasopharyngeal	100
NSCLC	99
Ovarian	83
Prostate	79
Pancreatic	81
RCC	84

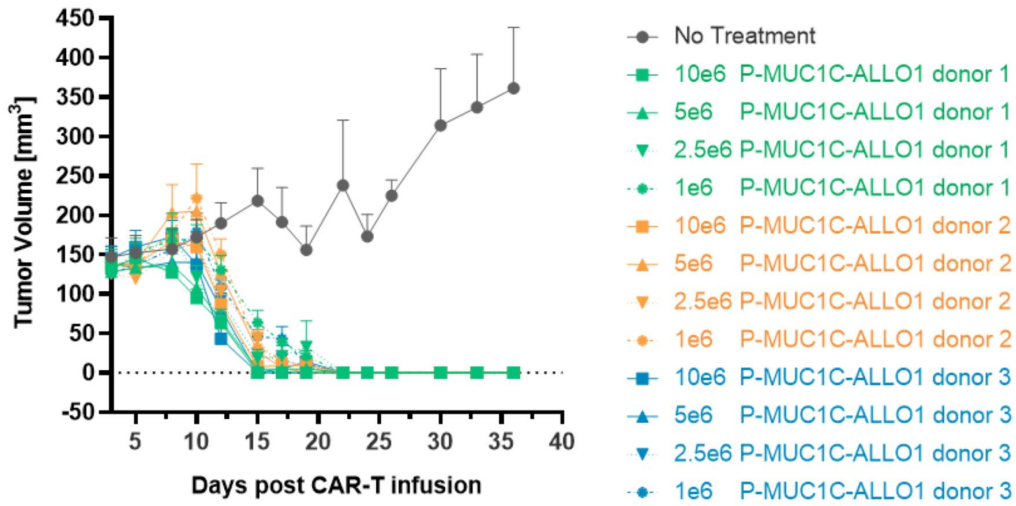
In our preclinical studies, an autologous MUC1-C CAR-T showed robust anti-tumor activity against multiple tumor lines:



The autologous MUC1-C CAR-T was evaluated for specificity and function using a standard T cell degranulation assay. Degranulation is a surrogate of T cell killing that can be easily measured by FACS staining for intracellular CD107a expression following co-culture with cells expressing target antigen. mRNA encoding the MUC1-C CAR-T was delivered to pan T cells via electroporation and the cells were rested overnight to allow for translation and surface expression of the CAR. T cells expressing the MUC1-C CAR-T were co-cultured for four to six hours with the indicated tumor cells. Six different cancer types were evaluated in these studies, including both solid and blood tumors. During the co-culture period, CD107a antibody was added to detect degranulation of T cells. The percentage of CD107a T cells is shown in the graph above and indicates tumor-specific activity. Degranulation frequency correlated highly with MUC1C expression on the target tumor cells.

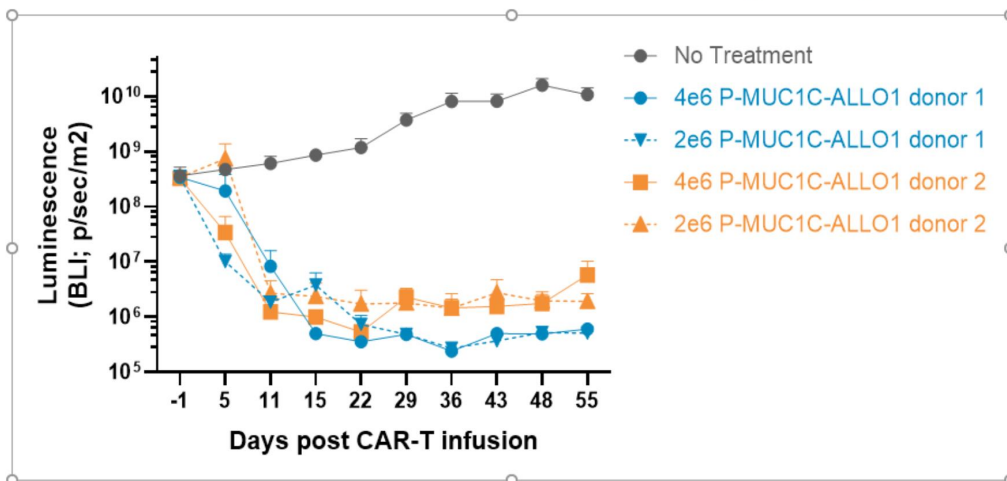
We tested P-MUC1C-ALLO1 in a preclinical xenograft model of triple-negative breast cancer in which immune deficient mice were implanted subcutaneously with a human MDA.MB.468 triple-negative breast cancer cell line. In this model, P-MUC1C-ALLO1 eliminated tumor cells to undetectable levels in both a standard and low dose arm, as shown below:

P-MUC1C-ALLO1 IN BREAST CANCER MODEL (MDA.MB.468)



We also tested P-MUC1C-ALLO1 in a preclinical xenograft model of ovarian cancer in which immunocompromised (NSG) mice were implanted intraperitoneally with 5×10^6 human OVCAR3 ovarian cancer cells 14 days before CAR-T cell injection. In this model, intraperitoneally administered P-MUC1C-ALLO1 at both a near standard dose (4×10^6) and a low stress dose (2×10^6) eliminated tumor cells to levels below the limit of detection, as shown in the figure below:

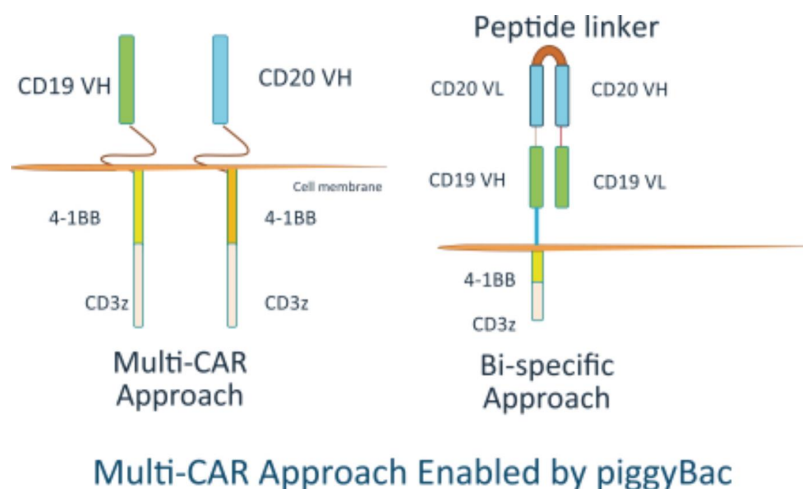
P-MUC1C-ALLO1 IN OVARIAN CANCER MODEL (OVCAR3)



We anticipate an IND filing and initiation of a Phase 1 clinical trial for an allogeneic version of a MUC1-C CAR, which we refer to as P-MUC1C-ALLO1, by the end of 2021. P-MUC1C-ALLO1 was designed to leverage the learnings of our P-BCMA-ALLO1 program.

Dual CAR-T Allogeneic Program Candidates

The very large cargo capacity of piggyBac allows for the inclusion of much larger or more therapeutic transgenes compared to viral-based technologies. We believe that our ability to include two or more fully functional CAR and/or TCR molecules into a T cell could be a significant competitive advantage. Unlike some competitors that have tried to use a bi-specific or tandem binder to approach this problem, we believe that including two, or more, full CAR or TCR molecules has the potential to be a more effective approach.



Dual CAR CD19/CD20. Dual CAR CD19/CD20 is an allogeneic, off-the-shelf CAR-T product candidate in preclinical development for B cell leukemia and lymphoma indications and some autoimmune diseases. Dual CAR CD19/CD20 contains two fully functional CAR molecules to target cells that express either CD19 or CD20. We believe that by targeting both CD19 and CD20, we have the potential to overcome some of the issues of earlier generation CD19 CAR-T products where antigen escape has been observed. We intend to develop this product candidate to be fully allogeneic by applying our learnings from the P-BCMA-ALLO1 program. We anticipate an IND filing in 2022.

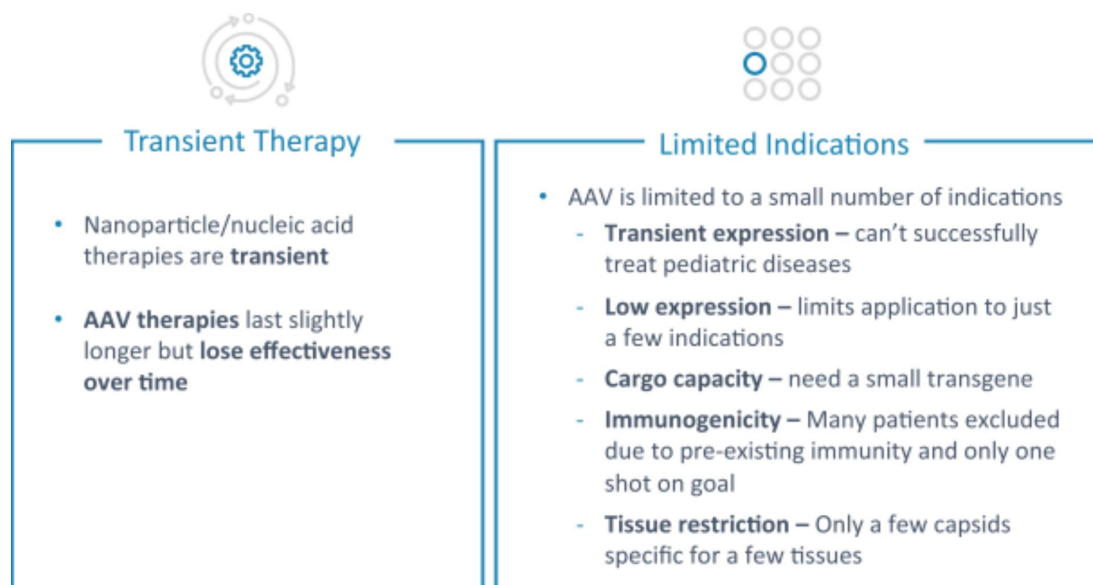
Dual CAR BCMA/CD19. Dual CAR BCMA/CD19 is an allogeneic, off-the-shelf CAR-T product candidate in preclinical development for multiple myeloma. Dual CAR BCMA/CD19 contains two fully functional CAR molecules to target cells that express either BCMA or CD19. Based on published studies of CD19 therapeutic candidates in multiple myeloma patients, we believe that targeting both BCMA and CD19 may be more effective than targeting BCMA alone in some patients because it has been hypothesized that there could be myeloma stem cells that express CD19 but do not express BCMA. In addition, including CD19 may prevent anti-drug antibody responses that could shorten the effectiveness of a BCMA-only therapy in some patients. We intend to develop this product candidate to be fully allogeneic by applying our learnings from the P-BCMA-ALLO1 program. We anticipate an IND filing after analyzing preliminary results observed in the P-BCMA-ALLO1 Phase 1 clinical trial.

Dual CAR (undisclosed). Dual CAR (undisclosed) is an allogeneic, off-the-shelf CAR-T product candidate in preclinical development for solid tumors. Dual CAR (undisclosed) contains two fully functional CAR molecules to target cells that express either of two targets that are currently undisclosed. We intend to develop this product candidate to be fully allogeneic by applying our learnings from the P-BCMA-ALLO1 program. We anticipate an IND filing after analyzing preliminary results observed in the more advanced Dual CAR programs.

Liver Directed Gene Therapy

The concept of *in vivo* gene therapy arose during the early 1970's, with initial human testing beginning in 1980. However, early clinical failures held back the development of the field and associated funding and progress was slow until the last decade. Within the last decade, gene therapy has expanded and gained more acceptance. Due to some clinical successes and associated funding and merger and acquisition activity, the field is now emerging as a major focus of new therapeutic development. Despite this re-emergence of interest and development, much of the *in vivo* gene therapy work faces significant challenges.

Among the primary limitations of most current gene therapies are the fact that these therapies are generally transient in nature and, therefore, limited to a narrow range of indications. These limitations are driven by a number of factors associated with using AAV as the standard method of delivering the therapeutic transgene. First, specific AAV capsids can be used to effectively infect a number of cell types *in vivo*, but AAV does not generally integrate into the genome without the virus' rep gene, which is removed in gene therapy applications to accommodate the therapeutic transgene. The lack of integration results in low expression levels of the therapeutic transgene that generally decrease over time. As cells divide, expression is eventually lost, thus making it difficult or impossible to use AAV-mediated gene therapies in rapidly dividing tissues, such as the pediatric liver. Unfortunately, the pediatric liver is the tissue that needs to be targeted in order to treat many monogenetic inborn errors of metabolism, particularly in the majority of patients that are more severely affected. Second, AAV has a relatively small cargo capacity, which can limit its ability to treat indications where a larger therapeutic transgene is needed to correct the underlying disease. The relatively small cargo capacity also limits the inclusion of additional features, such as larger tissue-specific promoters, insulators or safety switches. Third, AAV itself can be immunogenic with pre-existing antibodies in some patients. Furthermore, AAV-based therapies often elicit antibody-based immune reactions, making repeat dosing very challenging. Finally, earlier-generation AAV therapies require relatively high doses of virus to deliver enough of the gene to have a clinical effect, which creates safety issues associated with the AAV itself.



Our technology is designed to address the shortcomings of other AAV approaches in several important ways. First, by combining our piggyBac technology with AAV, we believe we can create a therapeutic that integrates the therapeutic transgene into the DNA and becomes a stable part of the patient's DNA, even in rapidly dividing cells. This results in the potential for single-treatment cures, even when treating indications that manifest predominantly in the pediatric liver. Second, piggyBac is highly efficient at integrating into DNA, resulting in stable and high expression levels of therapeutic transgenes even at relatively low doses, which we believe may allow potent activity in indications that are not currently treatable with AAV-only technologies. Furthermore, piggyBac in combination

with AAV might be effective at much lower viral doses when compared with AAV-only technologies and would therefore mitigate some of the risk of toxicity due to AAV itself.

We are also combining our piggyBac technology with our nanoparticle technology to deliver therapeutic transgenes in an effort to eliminate the need for AAV altogether. This would completely avoid virus-related toxicity and also enable delivery of larger genes and repeat dosing, which would further expand the number of indications that could be treated.

While our technology platforms enable the development of *in vivo* gene therapies in a wide array of applications, we are focusing our initial efforts on liver-directed gene therapy, where we have promising preclinical data and believe we have a significant competitive advantage over early generation gene therapies. We believe that our technology has the potential to address indications and patient populations that AAV-only technologies will not be able to address. In some cases, we believe that by combining our piggyBac technology with AAV or nanoparticle delivery, we have the potential to transform those transient therapies into single-treatment, lifetime durable responses.



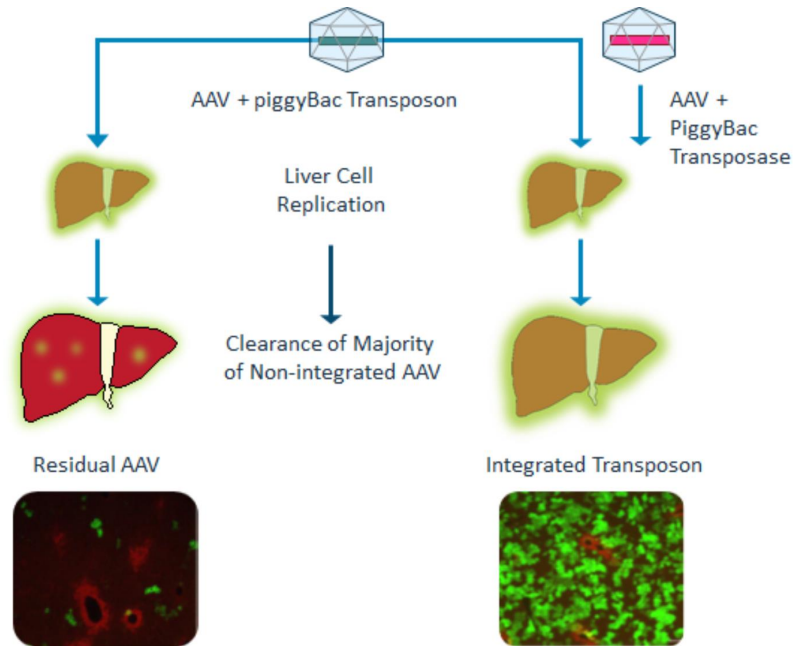
Single Treatment Cures

- **piggyBac permanently and stably integrates into DNA** resulting in the potential for **long-term stable expression**
- **piggyBac has the potential to work in juvenile liver** without diluted expression over time

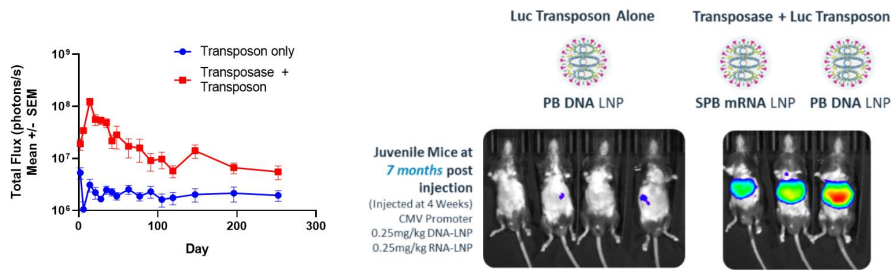
Countless Indications

- piggyBac has the potential to overcome the limitations of other systems
 - **High activity in virtually any cell type** enabling broad applicability
 - Results in **normal (wild type or higher) protein expression levels**
 - **Very large cargo capacity**
 - **No to low immunogenicity observed**
 - Ability to deliver piggyBac to many different cells and tissues both *ex vivo* and *in vivo*

Any AAV-based system can be converted into a piggyBac-AAV vector by simply adding the piggyBac ITRs, which can be as small as 50 base pairs each, inside of the AAV ITRs (AAV + piggyBac transposon). We expect this vector will perform the same as a standard AAV vector in the absence of the piggyBac transposase, which can be delivered in a second AAV (AAV + piggyBac transposase). When using an enhanced green fluorescent protein (EGFP) reporter gene as a surrogate for a therapeutic transgene and injecting the AAV + piggyBac transposon (no transposase) into animals, we observed a low level of EGFP expression in the liver of the mouse (lower left panel). Similar to other standard AAV therapies, there was a low expression level due to episomal (non-integrated) AAV and as such, it diminished over time, especially as the cells divided. However, when the AAV + piggyBac transposon was co-injected with the AAV + transposase, we observed a high, stable level of expression in a majority of hepatocytes, as shown in the lower right panel. In this case, the piggyBac transposase pulled the transgene out of the transposon and stably integrated it into the genome. As the cells divided, they replicated the integrated therapeutic transgene so all progeny cells permanently expressed it. This strategy has been used in three separate mouse models of various severe congenital liver genetic diseases: OTC deficiency, citrullinemia Type I and progressive familial intrahepatic cholestasis Type III, demonstrating the potential for single-treatment cures in each case.



One of the goals for our gene therapy programs is to be able to deliver our gene engineering technologies by nanoparticle to eliminate the need to use AAV due to its limitations. In preclinical work, we are seeing positive results in delivering piggyBac transposon (DNA) and piggyBac transposase (RNA) into animal models, resulting in significant integration and transgene expression in all zones of the liver. The following figure represents an experiment where we co-administered piggyBac transposon (DNA) and piggyBac transposase (RNA) formulated into separate nanoparticles to a juvenile mouse and measured levels of expression of a reporter gene in the liver out to 7 months. These data, while preliminary, potentially represent a significant step forward toward our goal of nanoparticle delivery of piggyBac, which we believe would represent a significant advance compared to traditional gene therapy.



Our Gene Therapy Programs

P-OTC-101

Overview

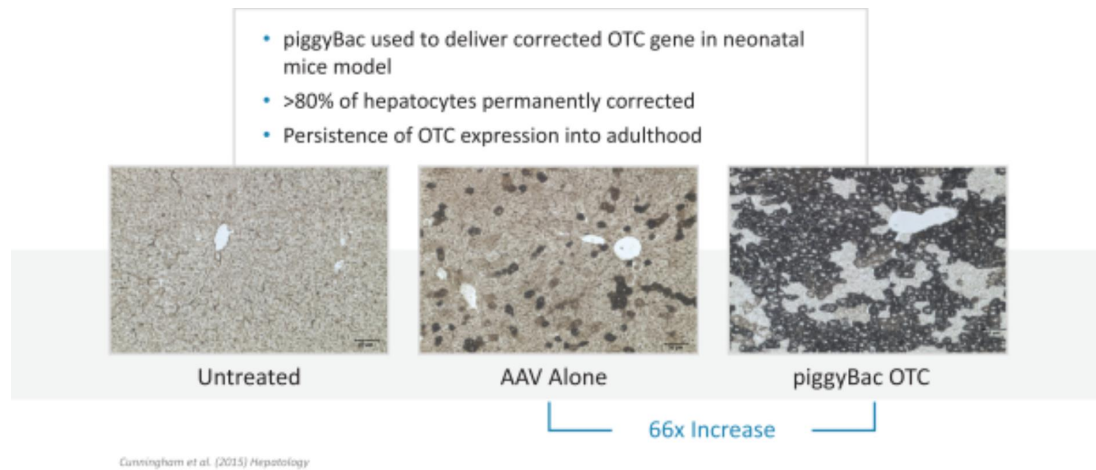
P-OTC-101 is an *in vivo* liver-directed gene therapy candidate for the treatment of OTC deficiency, which we believe has the potential to achieve single-treatment, lifetime durable responses. We are using our proprietary piggyBac DNA Delivery System combined with a liver-directed AAV to deliver a replacement OTC gene to the liver. P-OTC-101 is undergoing preclinical development and we anticipate an IND filing and initiation of a Phase 1 clinical trial in 2022.

Target Indication

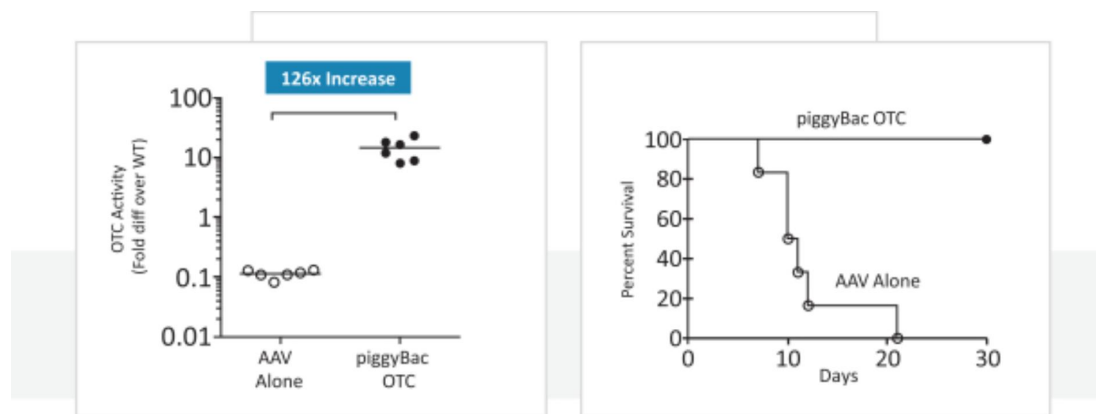
OTC is a rare genetic disorder characterized by complete or partial lack of the enzyme OTC. OTC is an enzyme that plays a role in the breakdown and removal of nitrogen from the body, a process known as the urea cycle. The lack of the OTC enzyme results in excessive accumulation of nitrogen in the form of ammonia (hyperammonemia) in the blood. Excess ammonia, which is a neurotoxin, travels to the central nervous system through the blood, resulting in symptoms of lethargy, vomiting, irritability and, in more severe cases, decreased muscle tone, seizures, enlarged liver, respiratory difficulties and death. A severe form of the disorder affects some infants, typically newly born males. A milder form of the disorder affects some children later in infancy. More severe forms of OTC comprise a high unmet medical need.

Preclinical Data

In preclinical studies conducted by our academic collaborators, the approach of combining piggyBac with AAV (piggyBac OTC) demonstrated stable and high level expression of a therapeutic transgene in the mouse liver compared to AAV-only technologies.



In the same study, treatment with piggyBac OTC resulted in a 126-fold increase in OTC levels compared with AAV alone and survival of all the animals in the treated group versus 0% survival in the AAV alone group. The expression of OTC at more than 10 times the normal (WT) levels also highlights the potential to lower the dose of piggyBac-OTC compared with standard AAV-alone therapies and the ability to still achieve single-treatment, durable responses, which would have additional cost and tolerability benefits compared to standard AAV therapies.



We have repeated preclinical studies with improved liver-specific AAV capsids and constructs, and selected a final P-OTC-101 product to begin manufacturing for clinical trials. We anticipate an IND filing and initiation of a Phase 1 clinical trial in 2022.

P-FVIII-101

Overview

P-FVIII-101 is a liver-directed gene therapy combining piggyBac technology with our nanoparticle delivery technology for the *in vivo* treatment of Hemophilia A.

We are using our proprietary piggyBac DNA Delivery System combined with our proprietary nanoparticle technology to deliver a Factor VIII therapeutic transgene. Preclinical studies are ongoing that will inform the development plan and timeline to IND.

Target Indication

Hemophilia A is a bleeding disorder caused by a deficiency in Factor VIII production with a high unmet need. Disease can range in severity from mild to severe and Factor VIII levels are correlated with the severity of the disease.

Preclinical Data

Our preclinical data demonstrates an ability to correct Factor VIII deficiency to normal levels in a juvenile mouse model using nanoparticle delivery of our P-FVIII-101 potential product candidate.

P-MMUT-101

Overview

P-MMUT-101 is an *in vivo* liver-directed gene therapy for the treatment of MMA due to a MMUT deficiency, or a defect in the MMUT gene, which we believe has the potential to achieve single-treatment, lifetime durable responses.

We are using our proprietary piggyBac DNA Delivery System combined with a liver-directed AAV to deliver a replacement MMUT gene with a P-MMUT-101 therapeutic transgene.

Due to uncertainty associated with the pandemic and the emergence of our Factor VIII program, we have slowed this program pending a re-evaluation following more data on nanoparticle delivery.

Target Indication

MMA is an inherited inborn error of metabolism in which the body is unable to process certain proteins and fats properly. The effects of MMA, which usually appear in early infancy, vary from mild to life-threatening. Affected infants can experience vomiting, dehydration, weak muscle tone, developmental delay, excessive tiredness, an enlarged liver, and failure to gain weight and grow at the expected rate. Long-term complications can include feeding problems, intellectual disability, chronic kidney disease, and inflammation of the pancreas. Without treatment, this disorder can lead to coma and death in some cases.

Preclinical Data

P-MMUT-101 is undergoing additional preclinical development.

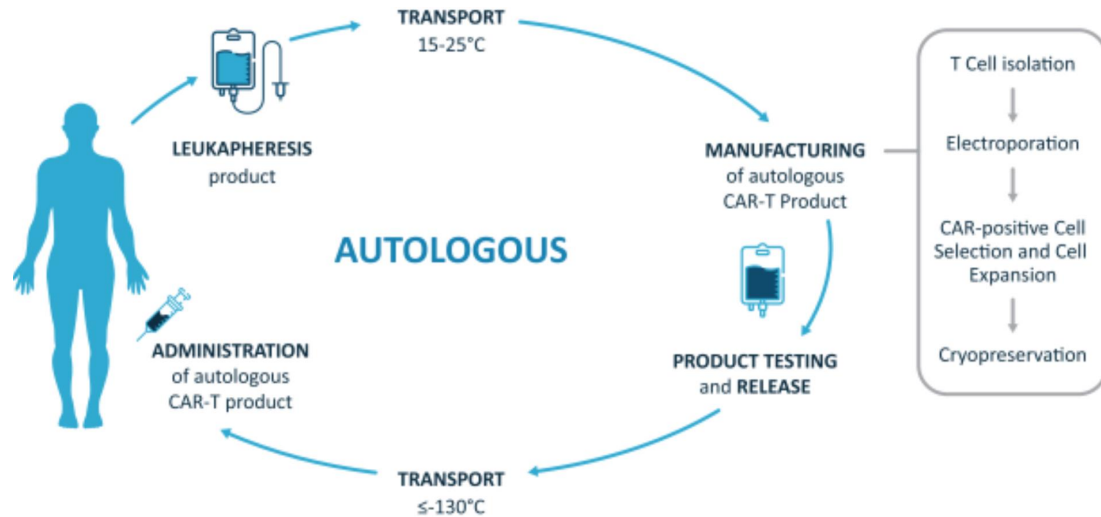
Potential Additional Programs and Partnership Opportunities

While we have leveraged our platform technologies to currently pursue the development of CAR-T and liver-directed gene therapy product candidates, our technologies have broad applicability across a wide array of cell and gene therapeutic modalities and diseases. Beyond the current pipeline, we and our collaborators have preclinical data that illustrate future potential applications of the technology platforms when combined in various ways. We may in the future use these tools to create T cell-based products to address indications beyond oncology, such as autoimmune diseases, infectious diseases, allergy-related diseases or even neurodegenerative diseases. CAR-T may also be used as an alternative and non-myeloablative preconditioning regimen for stem cell transplants. Our technologies also work well in other cell types and tissues including induced pluripotent stem cells, natural killer cells, HSCs, B cells, hepatocytes, muscles and many others, which could enable additional approaches for future therapeutics in a variety of indications. Lastly, we could use our Cas-CLOVER technology directly *in vivo*, similar to the approaches taken by other gene editing companies.

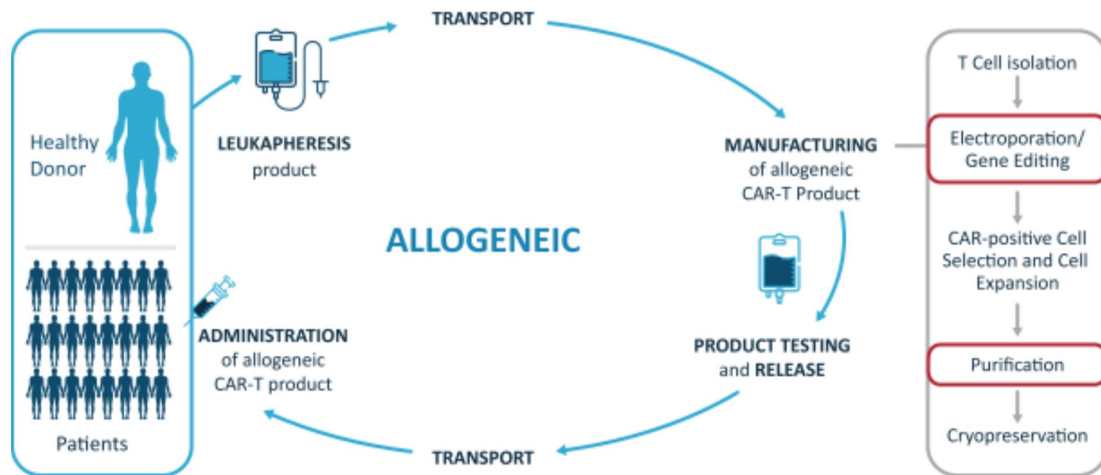
Our CAR-T Manufacturing Processes

Our autologous CAR-T product candidates consist of patient T cells that have been genetically engineered to express a CAR molecule and other genes. PBMCs are harvested by a standard leukapheresis procedure at the enrolling hospital, with the leukapheresis cells transported to the manufacturing site immediately subsequent to the procedure.

Manufacturing of autologous CAR-T product candidates includes CD4-positive and CD8-positive T cell isolation via positive selection, electroporation of the piggyBac DNA transposon transgene (encoding the CAR molecule gene, the DHFR positive selection gene and the safety switch gene) and Super piggyBac transposase RNA (the enzyme that mobilizes the piggyBac transposon transgene), CAR-positive T cell selection via methotrexate, and cell expansion. The final product is then bagged and cryopreserved. Following product release for administration, cryopreserved product candidates are shipped by courier to the pharmacy or applicable cell therapy facility of the enrolling study center where they are stored until the time of administration.



The manufacturing process for our allogeneic product candidates is nearly identical to the process for our autologous product candidates, except for the gene editing, a related additional purification step and the addition of booster molecules:



CAR-T Contract Manufacturing

We work with a number of third-party contract manufacturers for production of our product candidates. For the manufacturing of P-BCMA-101 we currently have relationships with two global contract manufacturing companies, Lonza Group and WuXi AppTec, Inc., from which we receive clinical supplies and on which we may rely on for commercial manufacturing. For the P-PSMA-101 Phase 1 clinical trial, we are utilizing C3i, a contract manufacturer in Montreal, Quebec affiliated with the University of Montreal Hospital. For our other product candidates, we are evaluating various third-party manufacturers for clinical supply. We also work with a variety of suppliers to provide our manufacturing raw materials and we currently source our media from Stemcell and DNA components from Aldebron. We believe that our relationships with our contract manufacturers and suppliers are good.

We also have an internal pilot GMP manufacturing facility in San Diego adjacent to our headquarters to develop and manufacture preclinical materials and clinical supplies for Phase 1 and Phase 2 clinical trials. We commenced operations in this facility in the second half of 2020. We plan to use the pilot facility initially for manufacturing our P-MUC1C-ALLO1 program. In the future, we may also build one or more commercial manufacturing facilities for any approved product candidates.

Commercialization Plans

We possess global rights to our product candidates and discovery programs. We intend to retain significant development and commercialization rights to our product candidates and, if marketing approval is obtained, to commercialize our product candidates on our own, or potentially with a partner, in the United States and other regions. We currently have no sales, marketing, or commercial product distribution capabilities and have no experience as a company in marketing products. We plan to build the necessary infrastructure and capabilities over time in the United States, and potentially other regions, following further advancement of our product candidates. Clinical data, the size of the addressable patient population, the size of the commercial infrastructure and manufacturing needs may all influence or alter our commercialization plans.

Competition

The biotechnology industry, and specifically the CAR-T and gene therapy sciences, are characterized by intense and rapidly changing competition to develop new technologies and proprietary products. While we believe that our proprietary approach and scientific expertise in CAR-T and gene therapies provide us with competitive advantages, we face potential competition from many different sources, including larger and better-funded pharmaceutical companies, as well as academic and research institutions. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are more effective, safer, have fewer or less severe side effects, are more convenient, or cost less than any products that we may develop. The key competitive factors affecting the success of our programs are likely to be their efficacy, safety, convenience and cost.

There are other organizations currently working toward commercializing existing therapies and/or new therapies for our initially selected indications. If these efforts are successful and their product candidates are approved or marketed prior to ours, it is possible they may increase the barriers to adoption of our product candidates.

Due to the promising clinical therapeutic effect of CAR-T product candidates in clinical trials, we anticipate direct competition from other organizations developing advanced T cell therapies and other types of oncology therapies. This would include companies in the CAR-T space including: Adaptimmune Therapeutics plc, Allogene, Inc., Astellas Pharma, Inc., Autolus Ltd., Bellicum Pharmaceuticals Inc., Bluebird Bio, Inc., Cellectis S.A., Janssen Pharmaceuticals Inc., Juno Therapeutics, Inc. (acquired by Celgene Corporation, now a Bristol-Meyers Squibb Company), Kite Pharma, Inc. (a Gilead Sciences, Inc. company), Legend Biotech Corporation, Novartis AG and Takeda Inc.

Immunotherapy and gene therapy approaches are further being pursued by several smaller biotechnology companies as well as larger pharmaceutical companies. We also face competition from non-cell-based treatments offered by companies such as Amgen Inc., AstraZeneca plc, Bristol-Myers Squibb Company, F. Hoffman-La Roche AG, GlaxoSmithKline plc, Merck & Co., Inc. and Pfizer Inc. Many of our competitors, either alone or with their collaboration partners, have substantially greater financial, technical and other resources, such as larger research and development staff and/or greater expertise in research and development, manufacturing, preclinical testing and conducting clinical trials.

Recent approvals and M&A activity have also spurred the creation of many companies now pursuing gene therapy technologies and indications. The landscape is evolving rapidly and these companies are too numerous to list, but would include companies such as Alnylam Pharmaceuticals, Inc., Astellas, Beam Therapeutics, Inc., BioMarin Pharmaceuticals, Inc., Bluebird Bio, Cellectis, CRISPR Therapeutics, AG, Editas Medicines, Inc., F. Hoffman-La Roche AG (acquired Spark Therapeutics, Inc.), Generation Bio, Inc., Intellia Therapeutics, Inc., LogicBio Therapeutics, Inc, Moderna, Inc., Novartis AG (acquired AveXis, Inc.), Passage Bio, Inc., Sangamo Therapeutics, Inc., Sarepta Therapeutics, Inc. and Ultragenyx, Inc.

In addition, smaller or early-stage companies may compete with us through collaborative arrangements with more established companies. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these enterprises. Mergers and acquisitions in the pharmaceutical, biotechnology and gene therapy industries are prevalent and may result in even more resources being concentrated among a smaller number of our competitors. Our competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials.

Intellectual Property

Intellectual property is of vital importance in our field and in biotechnology generally. We seek to protect and enhance proprietary technology, inventions, and improvements that are commercially important to the development of our business by seeking, maintaining, and defending patent rights, whether developed internally, acquired or licensed from third parties. We will also seek to rely on regulatory protection afforded through orphan drug designations, inclusion in expedited development and review, data exclusivity, market exclusivity and patent term extensions where available.

Our intellectual property estate is designed to provide multiple layers of protection, including: (1) patent rights with claims directed to platform technologies; (2) patent rights with claims directed to core components used in our products; (3) patent rights covering specific products; (4) patent rights covering methods of treatment for therapeutic indications; (5) patent rights covering methods of use for core components and platform technologies; and (6) patent rights covering innovative manufacturing processes. We also rely on trade secrets that may be important to the development of our business.

We believe our current layered patent estate, together with our efforts to develop and patent next generation technologies, provides us with substantial intellectual property protection.

We have filed or will file for patent protection in the United States and internationally for P-BCMA-101, P-PSMA-101, P-BCMA-ALLO1, P-PSMA-ALLO1, P-MUC1C-ALLO1, our Dual CAR product candidates, and P-OTC-101 and P-MMUT-101, our gene therapy product candidates. However, the area of patent and other intellectual property rights in biotechnology is an evolving one with many risks and uncertainties.

With respect to the platform technologies and core components described above (e.g., T_{SCM} compositions and manufacturing method, genetically-modified HSC manufacturing method, inducible safety switch, piggyBac DNA Delivery System, Cas-CLOVER gene editing technology, booster molecules for enhanced immune cell expansion, armoring strategies, and nanoparticle delivery methods) the intellectual property estate is comprised predominantly of company-owned or company-acquired intellectual property. We expect to file additional patent applications in support of current and new product candidates as well as new platform and core technologies. Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection of our current and future product candidates and the methods used to develop and manufacture them, as well as successfully

defending these patents against third-party challenges and operating without infringing on the proprietary rights of others. Our ability to stop third parties from making, using, selling, offering to sell or importing our products depends on the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities. We cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our product candidates, discovery programs and processes. For this and more comprehensive risks related to our intellectual property, please see the section titled “Risk Factors—Risks Related to Our Intellectual Property.”

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, including the United States, the patent term is 20 years from the earliest date of filing a non-provisional patent application. In the United States, a patent’s term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier filed patent or delays on the part of a patentee. In the United States, the patent term of a patent that covers an FDA-approved drug may also be eligible for patent term extension, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Hatch-Waxman Act permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. Patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to an approved drug may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our products receive FDA approval, we expect to apply for patent term extensions on patents covering those products. We plan to seek patent term extensions to any of our issued patents in any jurisdiction where these are available, however there is no guarantee that the applicable authorities, including the FDA in the United States, will agree with our assessment of whether such extensions should be granted, and if granted, the length of such extensions. For more information regarding the risks related to our intellectual property, see the section titled “Risk Factors—Risks Related to Our Intellectual Property.”

In some instances, we submit patent applications directly with the USPTO as provisional patent applications. Provisional applications for patents were designed to provide a lower-cost first patent filing in the United States. Corresponding non-provisional patent applications must be filed not later than 12 months after the provisional application filing date. The corresponding non-provisional application benefits in that the priority date(s) of the patent application is/are the earlier provisional application filing date(s), and the patent term of the finally issued patent is calculated from the later non-provisional application filing date. This system allows us to obtain an early priority date, add material to the patent application(s) during the priority year, obtain a later start to the patent term and to delay prosecution costs, which may be useful in the event that we decide not to pursue examination in an application. While we intend to timely file non-provisional patent applications relating to our provisional patent applications, we cannot predict whether any such patent applications will result in the issuance of patents that provide us with any competitive advantage.

We file U.S. non-provisional applications and Patent Cooperation Treaty, or PCT, applications that claim the benefit of the priority date of earlier filed provisional applications, when applicable. The PCT system allows a single application to be filed within 12 months of the original priority date of the patent application, and to designate all of the 152 PCT member states in which national patent applications can later be pursued based on the international patent application filed under the PCT. The PCT searching authority performs a patentability search and issues a non-binding patentability opinion which can be used to evaluate the chances of success for the national applications in foreign countries prior to having to incur the filing fees. Although a PCT application does not issue as a patent, it allows the applicant to seek protection in any of the member states through national-phase applications. At the end of the period of two and a half years from the first priority date of the patent application, separate patent applications can be pursued in any of the PCT member states either by direct national filing or, in some cases by filing through a regional patent organization, such as the European Patent Organization. The PCT system delays expenses, allows a limited evaluation of the chances of success for national/regional patent applications and enables substantial savings where applications are abandoned within the first two and a half years of filing.

For all patent applications, we determine claiming strategy on a case-by-case basis. Advice of counsel and our business model and needs are always considered. We file patents containing claims for protection of all useful applications of our proprietary technologies and any products, as well as all new applications and/or uses we discover for existing technologies and products, assuming these are strategically valuable. We continuously reassess the number and type of patent applications, as well as the pending and issued patent claims to ensure that maximum coverage and value are obtained for our processes, and compositions, given existing patent office rules and regulations. Further, claims may be modified during patent prosecution to meet our intellectual property and business needs.

We recognize that the ability to obtain patent protection and the degree of such protection depends on a number of factors, including the extent of the prior art, the novelty and non-obviousness of the invention, and the ability to satisfy the enablement requirement of the patent laws. In addition, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted or further altered even after patent issuance. Consequently, we may not obtain or maintain adequate patent protection for any of our future product candidates or for our technology platform. We cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient proprietary protection from competitors. Any patents that we hold may be challenged, circumvented or invalidated by third parties.

In addition to patent protection, we also rely on trademark registration, trade secrets, know how, other proprietary information and continuing technological innovation to develop and maintain our competitive position. We seek to protect and maintain the confidentiality of proprietary information to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. Thus, we may not be able to meaningfully protect our trade secrets. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual during the course of the individual's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. Our agreements with employees also provide that all inventions conceived by the employee in the course of employment with us or from the employee's use of our confidential information are our exclusive property. However, such confidentiality agreements and invention assignment agreements can be breached and we may not have adequate remedies for any such breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting trade secrets, know-how and inventions. For more information regarding the risks related to our intellectual property, see the section titled "Risk Factors—Risks Related to Our Intellectual Property."

The patent positions of biotechnology companies like ours are generally uncertain and involve complex legal, scientific and factual questions. Our commercial success will also depend in part on not infringing upon the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent would require us to alter our development or commercial strategies, or our products or processes, obtain licenses or cease certain activities. Our breach of any license agreements or our failure to obtain a license to proprietary rights required to develop or commercialize our future products may have a material adverse impact on us. If third parties prepare and file patent applications in the United States that also claim technology to which we have rights, we may have to participate in interference or derivation proceedings in the USPTO to determine priority of invention. For more information, see the section titled "Risk Factors—Risks Related to Our Intellectual Property."

When available to expand market exclusivity, our strategy is to obtain, or license additional intellectual property related to current or contemplated development platforms, core elements of technology and/or product candidates.

Company-Owned Intellectual Property

P-BCMA-101 is covered by a number of filings, including a published PCT application filed in 2017 that entered the national phase in January of 2019. National phase applications are pending in several countries outside the United States, including most major market countries. Composition of matter claims issuing from this application would not expire before 2037.

P-PSMA-101 is covered by a number of filings, including, a published PCT application filed in March 2019 that entered the national stage in September of 2020. National phase applications are pending in several countries outside the United States, including most major market countries. Composition of matter claims issuing from this application would not expire before 2039.

P-BCMA-ALLO1 is covered by a number of filings, including, a published PCT application filed in December 2018 that entered the national stage in June of 2020. National phase applications are pending in several countries outside the United States, including most major market countries. Composition of matter claims issuing from this application would not expire before 2038.

P-MUC1C-ALLO1 is covered by a number of filings, including a published PCT application filed in 2017 that entered the national phase in January of 2019. National phase applications are pending in several countries outside the United States, including most major market countries. Composition of matter claims issuing from this application would not expire before 2037. In addition, an unpublished PCT application filed in September of 2020 will publish in March 2021. Composition of matter claims issuing from these applications would not expire before 2040.

Our Gene Therapy programs including P-OTC-101 and P-MMUT-101 are covered by a number of filings, including as of March 4, 2020, a pending provisional application that is due for conversion to a non-provisional application in March 2021. Composition of matter claims issuing from this application would not expire before 2041.

Our P-PSMA-ALLO1 and Dual CAR Programs, including Dual CAR CD19/CD20, Dual CAR BCMA/CD19 and Dual CAR (UD), are earlier in development and our intellectual property coverage is still being developed.

Core components of each of these product candidates are protected by company-owned platform applications directed to Centyrin binders (P-BCMA-101 and P-PSMA-101) or heavy-chain-only antibody fragment binders (P-BCMA-ALLO1), booster molecules for enhanced immune cell expansion (currently all allogeneic products), early memory T-cells (including T_{S_{CM}}) and methods of producing same (P-BCMA-101, P-PSMA-101, P-BCMA-ALLO1 and P-MUC1C-ALLO1), piggyBac transposition systems (all products), inducible safety switches (all products), marker genes for facilitating simultaneous selection and expansion of modified cells for product manufacture, and self-cleaving peptides for trivalent transposon constructs (all products). Notably in December 2019, we were issued a U.S. patent that has claims that cover any modified T cell product that has 25% or more T_{S_{CM}} cells and has a patent term expiring in 2037. We also have issued U.S. patents covering manufacturing methods and cell culture media used to produce these genetically modified T_{S_{CM}} cells that have patent terms expiring in 2037. We also have an issued composition of matter patent in the U.S. protecting our Cas-CLOVER site-specific gene editing system that has a patent term expiring in 2032. We also have issued composition of matter patents in the U.S. protecting our piggyBac DNA Delivery System that have patent terms expiring in 2030.

Acquired Intellectual Property

As a spin-out from Transposagen Biopharmaceuticals, Inc., or Transposagen, at inception, we acquired intellectual property related to piggyBac transposition systems and methods for use. This acquisition further comprised intellectual property related to next-generation gene editing systems and methods for use.

We acquired Vindico NanoBioTechnology, LLC (formerly known as Vindico NanoBioTechnology, Inc.) in October 2016. As part of this transaction, we acquired intellectual property related to polymer-based nanoparticle compositions and methods of use for delivery of, for example, gene therapy technologies.

License Agreements

License Agreement with Janssen Biotech

On August 3, 2015, we entered into a license agreement, or the Janssen Agreement, with Janssen Biotech Inc., or Janssen, pursuant to which we obtained an exclusive, sublicenseable, worldwide license to research, develop, manufacture and commercialize pharmaceutical products comprising autologous CAR-modified T-cells or any CAR-modified natural killer or CAR-modified natural-killer-like cells expressing certain Centyrin molecules, for the treatment or prevention of any disease in humans. We are obligated to use commercially reasonable efforts to develop and commercialize at least one such licensed product. We utilize these license rights in our P-BCMA-101 and P-PSMA-101 product candidates. Under the Janssen Agreement, we also have the right to engage with authorized third parties to screen Janssen's Centyrin library for agents that bind or modify targets of interest for our internal research and development purposes for potential use in a licensed product.

Pursuant to the Janssen Agreement, we paid Janssen an upfront fee of \$0.2 million. As of December 31, 2020, we have paid approximately \$3.3 million in milestone development fees relating to P-BCMA-101 and approximately \$0.7 million in milestone development fees relating to P-PSMA-101. We are required to pay Janssen up to an aggregate of \$75.8 million upon the achievement of certain clinical, regulatory and sales milestones for the first licensed product and up to an aggregate of \$46.8 million upon the achievement of certain clinical, regulatory and sales milestones for each licensed product thereafter. We are also obligated to pay, on a product-by-product and country-by-country basis, tiered royalties in the low single-digit percentage range on annual net sales, with the royalty rates varying depending on if there is a valid claim present within the licensed patent rights covering the licensed product in the applicable country in which the net sales occur. The royalty rates are also subject to reduction upon certain other events.

The Janssen Agreement will terminate on the last to expire royalty term, which is determined on a licensed product-by-licensed product and country-by-country basis, and is the later of (1) 10 years from the first commercial sale of the licensed product in the country, (2) the last to expire valid claim within the licensed patent in the country or (3) expiry of regulatory exclusivity granted by the prevailing governmental authority for the licensed product in the country. We also have the right to terminate the Janssen Agreement in its entirety or on a licensed product-by-licensed product basis, for any reason upon 60 days prior written notice to Janssen. Either party may terminate the Janssen Agreement upon a material breach by the other party that is not cured within 60 days after receiving written notice, or upon giving written notice within 30 days of the other party's bankruptcy. If we terminate the Janssen Agreement for convenience or Janssen terminates the Janssen Agreement due to our breach of our diligence obligations thereunder, Janssen will have an option to negotiate a license from us to research, develop and commercialize the Centyrin CAR molecules and/or Centyrin therapeutic molecules. If Janssen exercised this option, Janssen would be obligated to pay us a fee in the low six figure dollar range.

April 2017 Commercial License Agreement with TeneoBio

On April 27, 2017, we entered into a commercial license agreement, or the 2017 TeneoBio Agreement, with TeneoBio, Inc., or TeneoBio, pursuant to which we obtained an exclusive, sublicenseable, worldwide license to use and develop pharmaceutical products comprising allogeneic T-cells expressing a CAR molecule containing certain heavy-chain-only sequences provided by TeneoBio (a CAR containing a non-naturally occurring heavy-chain-only antibody fragment) for the treatment of human disease. We utilize these license rights in our P-BCMA-ALLO1 product candidate.

Pursuant to the 2017 TeneoBio Agreement, we have paid TeneoBio \$0.5 million through our selection of the antibodies licensed under the 2017 TeneoBio Agreement. We are required to pay TeneoBio up to an aggregate of \$20.5 million upon the first achievement of certain clinical and regulatory milestones for any allogeneic product and up to an aggregate of \$20.5 million upon the first achievement of certain clinical and regulatory milestones for any autologous product. We are also obligated to pay, on a product-by-product and country-by-country basis, a royalty in the low single-digit percentage on net sales of all licensed products.

The 2017 TeneoBio Agreement will terminate on the last to expire valid claim of the licensed patents in all countries. We may also terminate the 2017 TeneoBio Agreement at any time upon 60 days prior written notice to

TeneoBio. Either party may terminate the 2017 TeneoBio Agreement upon a material breach by the other party that is not cured within 90 days after receiving written notice of the breach, or upon a bankruptcy of the other party.

August 2018 Commercial License Agreement with TeneoBio

On August 3, 2018, we entered into a commercial license agreement, or the 2018 TeneoBio Agreement, with TeneoBio for the development and use of TeneoBio's human heavy-chain-only antibodies in CAR-T cell therapies. Under the terms of the 2018 TeneoBio Agreement, we have the option to obtain exclusive rights to research, develop and commercialize up to a certain number of targets from TeneoBio.

Pursuant to the 2018 TeneoBio Agreement, we paid TeneoBio an upfront fee of \$4.0 million. We are required to pay additional fees in the low to mid six figure dollar range upon (1) selecting exclusivity for a particular target, which restricts TeneoBio from licensing that particular target to a third party for a period of time, (2) continuing exclusivity for any selected target on each anniversary thereafter and (3) exercising our commercial option for each target. We are required to pay TeneoBio up to an aggregate of \$31.0 million upon the first achievement of certain clinical and regulatory milestones for each product. We are also obligated to pay, on a product-by-product and country-by-country basis, a low single-digit percentage royalty on net sales of any licensed products. The royalty rate is subject to reduction upon certain events.

The 2018 TeneoBio Agreement will terminate on the last to expire valid claim of the licensed patents in all countries. We may also terminate the 2018 TeneoBio Agreement with respect to one or more targets at any time upon 60 days prior written notice. Either party may terminate the 2018 TeneoBio Agreement upon a material breach by the other party that is not cured within 90 days after receiving written notice of the breach, or upon a bankruptcy of the other party.

License Agreement with Genus Oncology

On October 24, 2019, we entered into a license agreement, or the Genus Agreement, with Genus Oncology, LLC, or Genus. Pursuant to the Genus Agreement, we paid Genus an upfront fee of \$1.5 million and Genus granted us the option, which was exercised for an additional \$1.5 million in April 2020, to obtain an exclusive, sublicenseable, worldwide license under certain patents and a non-exclusive, sublicenseable, worldwide license under certain know-how controlled by Genus to research, develop and commercialize pharmaceutical products incorporating CAR cells expressing antibodies and derivatives thereof targeting MUC1, or a Genus licensed product, and a non-exclusive, sublicenseable, worldwide license under certain patents and know-how controlled by Genus to research, develop and commercialize companion diagnostics for the treatment, prevention and palliation of human diseases and conditions. The licenses granted pursuant to the Genus Agreement are subject to certain rights retained by an upstream licensor and the rights of the U.S. government. The retained rights of the upstream licensor pertain only to the ability of the upstream licensor to conduct teaching, education and other non-commercial research activities in the licensed field and for other academic, governmental or not-for-profit organizations to conduct non-commercial research activities in the licensed field, and do not limit our ability to pursue our programs and product candidates. We may use a Genus antibody or derivative thereof targeting MUC1 as a binder in our P-MUC1C-ALLO1 product candidate. Multiple other aspects of our P-MUC1C-ALLO1 product candidate are covered by other patents and intellectual property that we own or license and are not subject to rights of the U.S. government.

Pursuant to the Genus Agreement, we are also required to pay Genus up to an aggregate of \$71.0 million upon first achievement of certain clinical, regulatory and sales milestones for any Genus licensed product and companion diagnostics. We are also obligated to pay, on a product-by-product and country-by-country basis, tiered royalties in the low to mid-single-digit percentage on net sales of any Genus licensed products and related companion diagnostics, subject to certain customary reductions.

The Genus Agreement will expire on the last to expire royalty term, which is determined on a product-by-product and country-by-country basis, and is the later of (1) the last to expire valid claim within the licensed patents covering the Genus licensed product in the country, (2) expiration of regulatory exclusivity for the Genus licensed product in the country and (3) 10 years from the first commercial sale of the Genus licensed product in the country. We may also terminate the Genus Agreement at any time upon 30 days prior written notice to Genus. Either party

may terminate the Genus license agreement upon a material breach by the other party that is not cured within 90 days after receiving written notice of the breach. Genus also has the right to terminate the Genus Agreement immediately upon our bankruptcy or if we fail to initiate a Phase 1 clinical trial for a Genus licensed product within 20 months after approval of an IND submitted for such Genus licensed product.

License Agreement with HMGU

On May 20, 2016, we entered into a patent license agreement, or the HMGU License Agreement, with Helmholtz-Zentrum München—Deutsches Forschungszentrum für Gesundheit und Umwelt GmbH, or HMGU, pursuant to which we obtained exclusive worldwide rights to research, develop, manufacture and commercialize products and services claimed by certain patent applications and patents owned by HMGU covering the nuclease Clo051 in certain fields of use, including human pharmaceutical products. We utilize these license rights in our Cas-CLOVER gene editing technology including P-BCMA-ALLO1 and our other planned allogeneic programs.

Pursuant to the HMGU License Agreement, we paid HMGU an upfront fee of \$11,506, equal to €10,000 on the date of payment. We are required to pay HMGU annual maintenance fees credited against royalties due for the same year. We are also required to pay HMGU up to an aggregate of €1.7 million upon the first achievement of certain clinical and regulatory milestones for the first licensed product where Clo051 is part of the therapeutic agent and up to an aggregate of €0.9 million upon the first of certain clinical and regulatory milestones for the first licensed product where Clo051 is not part of the therapeutic agent. We are obligated to pay, on a licensed product-by-licensed product or licensed service-by-licensed service and country-by-country basis, royalties in the low single-digit percentage range on annual net sales, with the royalty rates varying depending on whether the licensed products are therapeutics or the licensed services are for therapeutic use and whether Clo051 is part of the therapeutic agent or used to generate the therapeutic agent. We currently use Clo051 as part of our gene engineering technology to generate our product candidates.

The HMGU License Agreement will terminate on the last to expire royalty term, which is determined on a licensed product-by-licensed product and country-by-country basis. We also have the right to terminate the HMGU License Agreement upon giving written notice within 3 months prior to the end of a calendar year. Either party may terminate the HMGU License Agreement upon a material breach by the other party that is not cured within six weeks after receiving written notice of the breach. The HMGU License Agreement terminates automatically if we become bankrupt.

Government Regulation

The FDA and other regulatory authorities at federal, state, and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring, and post-approval reporting of biologics such as those we are developing. We, along with third-party contractors, will be required to navigate the various preclinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates.

The process required by the FDA before biologic product candidates may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests and animal studies performed in accordance with the FDA's current Good Laboratory Practices regulation;
- submission to the FDA of an IND, which must become effective before clinical trials may begin and must be updated annually or when significant changes are made;
- approval by an independent Institutional Review Board, or IRB, or ethics committee at each treatment site before the trial is commenced;
- performance of adequate and well-controlled human clinical trials to establish the safety, purity and potency of the proposed biologic product candidate for its intended purpose;

- preparation of and submission to the FDA of a BLA after completion of all pivotal clinical trials;
- satisfactory completion of an FDA Advisory Committee review, if applicable;
- a determination by the FDA within 60 days of its receipt of a BLA to file the application for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the proposed product is produced to assess compliance with cGMP and to assure that the facilities, methods and controls are adequate to preserve the biological product's continued safety, purity and potency, and of selected clinical investigation sites to assess compliance with Good Clinical Practices, or GCP; and
- FDA review and approval of the BLA to permit commercial marketing of the product for particular indications for use in the United States.

Preclinical and Clinical Development

Prior to beginning the first clinical trial with a product candidate, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for clinical studies. The IND also includes results of animal and in vitro studies assessing the toxicology, pharmacokinetics, pharmacology, and pharmacodynamic characteristics of the product; chemistry, manufacturing, and controls information; and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises safety concerns or questions about the proposed clinical trial. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND therefore may or may not result in FDA authorization to begin a clinical trial. In addition to the submission of an IND to the FDA before initiation of a clinical trial in the United States, certain human clinical trials involving recombinant or synthetic nucleic acid molecules are subject to the FDA's oversight and other clinical trial regulations, and oversight at the local level as set forth in the NIH Guidelines. Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site and must monitor the study until completed. Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing clinical studies and clinical study results to public registries.

For purposes of BLA approval, human clinical trials are typically conducted in three sequential phases that may overlap.

- Phase 1—The investigational product is initially introduced into healthy human subjects or patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.
- Phase 2—The investigational product is administered to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.
- Phase 3—The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may be made a condition to approval of the BLA. Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the biological characteristics of the product candidate and must finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product, or for biologics, the safety, purity and potency. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

BLA Submission and Review

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, nonclinical studies and clinical trials are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. The BLA must include all relevant data available from pertinent preclinical and clinical studies, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls, and proposed labeling, among other things. The submission of a BLA requires payment of a substantial application user fee to FDA, unless a waiver or exemption applies, and the sponsor of an approved BLA is also subject to an annual program fee.

Once a BLA has been submitted, the FDA's goal is to review standard applications within ten months after it accepts the application for filing, or, if the application qualifies for priority review, six months after the FDA accepts the application for filing. In both standard and priority reviews, the review process is often significantly extended by FDA requests for additional information or clarification. The FDA reviews a BLA to determine, among other things, whether a product is safe, pure and potent and the facility in which it is manufactured, processed, packed, or held meets standards designed to assure the product's continued safety, purity and potency. The FDA may convene an advisory committee to provide clinical insight on application review questions. Before approving a BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more treatment sites to assure compliance with GCP. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates a BLA and conducts inspections of manufacturing facilities where the investigational product and/or its drug substance will be produced, the FDA may issue an approval letter or a Complete Response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A Complete Response letter will describe all of the deficiencies that the FDA has identified in the BLA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the Complete Response letter without first conducting required inspections, testing submitted product lots, and/or reviewing proposed labeling. In issuing the Complete Response letter, the FDA may recommend actions that the applicant might take to place the BLA in condition for approval, including requests for additional information or clarification. The FDA may delay or refuse approval of a BLA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with a Risk Evaluation and Mitigation Strategy, or REMS, to ensure the benefits of the product outweigh its risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a product and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. The FDA may require one or more Phase 4 post market studies and surveillance to further assess and monitor the product's safety and effectiveness after commercialization and may limit further marketing of the product based on the results of these post-marketing studies.

Expedited Development and Review Programs

The FDA offers a number of expedited development and review programs for qualifying product candidates. The fast track program is intended to expedite or facilitate the process for reviewing new products that meet certain criteria. Specifically, new products are eligible for fast track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a fast track product has opportunities for frequent interactions with the review team during product development and, once a BLA is submitted, the product may be eligible for priority review. A fast track product may also be eligible for rolling review, where the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA, the FDA agrees to accept sections of the BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA.

A product intended to treat a serious or life-threatening disease or condition may also be eligible for breakthrough therapy designation to expedite its development and review. A product can receive breakthrough therapy designation if preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the fast track program features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product, including involvement of senior managers.

Any marketing application for a biologic submitted to the FDA for approval, including a product with a fast track designation and/or breakthrough therapy designation, may be eligible for other types of FDA programs intended to expedite the FDA review and approval process, such as priority review and accelerated approval. A product is eligible for priority review if it has the potential to provide a significant improvement in the treatment, diagnosis or prevention of a serious disease or condition compared to marketed products. For products containing new molecular entities, priority review designation means the FDA's goal is to take action on the marketing application within six months of the 60-day filing date (compared with ten months under standard review).

Additionally, products studied for their safety and effectiveness in treating serious or life-threatening diseases or conditions may receive accelerated approval upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA will generally require the sponsor to perform adequate and well-controlled post-marketing clinical studies to verify and describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. While we currently intend to seek accelerated approval of P-BCMA-101 following the completion of our planned Phase 2 clinical trial, it is possible that at the time of a BLA submission, P-BCMA-101 would not be eligible for accelerated approval or the FDA could determine that accelerated approval is not warranted. In particular, because the FDA has already approved therapies for multiple myeloma, and because additional drugs may be approved for multiple myeloma while we are developing P-BCMA-101, it is difficult to predict whether accelerated approval will be possible for P-BCMA-101 at the time we expect to submit a BLA. The FDA has indicated that if data from our planned Phase 2 clinical trial do not provide evidence sufficient for accelerated approval, that additional clinical testing would be required to support approval, with a preference for us to conduct a randomized controlled trial or trials.

In 2017, FDA established a new regenerative medicine advanced therapy, or RMAT, designation as part of its implementation of the 21st Century Cures Act. The RMAT designation is intended to fulfill the 21st Century Cures Act requirement that FDA facilitate an efficient development program for, and expedite review of, any drug that meets the following criteria: (1) it qualifies as a RMAT, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions; (2) it is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and (3) preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such a disease or condition. Like breakthrough therapy designation, RMAT designation provides potential benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate and eligibility for rolling review and priority review. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion to additional sites. Once approved, when appropriate, the FDA can permit fulfillment of post-approval requirements under accelerated approval through the submission of clinical evidence, clinical studies, patient registries, or other sources of real-world evidence such as electronic health records; through the collection of larger confirmatory datasets; or through post-approval monitoring of all patients treated with the therapy prior to approval.

Fast track designation, breakthrough therapy designation, priority review, accelerated approval, and RMAT designation do not change the standards for approval but may expedite the development or approval process.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for this type of disease or condition will be recovered from sales in the United States for that drug or biologic. Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. The orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusive approval (or exclusivity), which means that the FDA may not approve any other applications, including a full BLA, to market the same biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. Orphan drug exclusivity does not prevent FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the BLA application fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

Post-Approval Requirements

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and promotion of the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing user fee requirements, under which FDA assesses an annual program fee for each product identified in an approved BLA. Biologic manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of a product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of existing product approvals;
- product seizure or detention, or refusal of the FDA to permit the import or export of products;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of biologics. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across

medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

Biosimilars and Reference Product Exclusivity

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, the ACA, signed into law in 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-approved reference biological product. To date, a number of biosimilars have been licensed under the BPCIA, and numerous biosimilars have been approved in Europe. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars.

Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. Complexities associated with the larger, and often more complex, structures of biological products, as well as the processes by which such products are manufactured, pose significant hurdles to implementation of the abbreviated approval pathway that are still being worked out by the FDA.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing that applicant's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

A biological product can also obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

The BPCIA is complex and continues to be interpreted and implemented by the FDA. In addition, recent government proposals have sought to reduce the 12-year reference product exclusivity period. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. As a result, the ultimate impact, implementation, and impact of the BPCIA is subject to significant uncertainty.

Other U.S. Healthcare Laws and Compliance Requirements

In the United States, our activities are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including but not limited to, the Centers for Medicare & Medicaid Services, or CMS, other divisions of the U.S. Department of Health and Human Services, or HHS (such as the Office of Inspector General and the Health Resources and Service Administration), the Department of Justice, or the DOJ, and individual U.S. Attorney offices within the DOJ, and state and local governments. For example, sales, marketing and scientific/educational grant programs may have to comply with the anti-fraud and abuse provisions of the Social Security Act, the false claims laws, the privacy and security provisions of the Health Insurance Portability and Accountability Act, or HIPAA, and similar state laws, each as amended, as applicable.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity, from knowingly and willfully offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any item or service reimbursable, in whole or in part, under Medicare, Medicaid or other federal healthcare programs. The term remuneration has been interpreted broadly to include anything of value. The federal Anti-Kickback Statute has been interpreted to apply to arrangements between therapeutic product manufacturers on one hand and prescribers and purchasers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution. The exceptions and safe harbors are drawn narrowly and practices that involve remuneration that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the federal Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. Our practices, including our arrangements with physicians, may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor.

Additionally, the intent standard under the federal Anti-Kickback Statute was amended by the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, ACA, to a stricter standard such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, the ACA codified case law that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act, or FCA.

The federal false claims and civil monetary penalty laws, including the FCA, which can be enforced by private citizens through civil qui tam actions, prohibit any person or entity from, among other things, knowingly presenting, or causing to be presented, a false or fraudulent claim for payment to, or approval by, the federal healthcare programs, including Medicare and Medicaid, or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. A claim includes "any request or demand" for money or property presented to the U.S. government. For instance, historically, pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies' marketing of the product for unapproved, off-label, and thus generally non-reimbursable, uses.

HIPAA created additional federal criminal statutes that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any money or property owned by, or under the control or custody of, any healthcare benefit program, including private third-party payors, willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up by trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Like the federal Anti-Kickback Statute, the ACA amended the intent standard for certain healthcare fraud statutes under HIPAA such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

Also, many states have similar, and typically more prohibitive, fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor. Additionally, to the extent that our product is sold in a foreign country, we may be subject to similar foreign laws.

We may be subject to data privacy and security regulations by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, impose requirements relating to the privacy, security and transmission of individually identifiable health information on certain healthcare providers, healthcare clearinghouses, and health plans, known as covered entities, and independent contractors, or agents of covered entities that receive or obtain individually identifiable health information in connection with providing a service on behalf of a covered entity, known as a business associates, as well as their covered subcontractors. Among other

things, HITECH makes HIPAA's privacy and security standards directly applicable to business associates. HITECH also created four new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorneys' fees and costs associated with pursuing federal civil actions. In addition, many state laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways, are often not pre-empted by HIPAA, and may have a more prohibitive effect than HIPAA, thus complicating compliance efforts.

In addition, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average sales price and best price. Further, these prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. It is difficult to predict how Medicare coverage and reimbursement policies will be applied to our products in the future and coverage and reimbursement under different federal healthcare programs are not always consistent. Medicare reimbursement rates may also reflect budgetary constraints placed on the Medicare program.

Additionally, the federal Physician Payments Sunshine Act, or the Sunshine Act, within the ACA, and its implementing regulations, require that certain manufacturers of drugs, devices, biological and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) report annually to CMS information related to certain payments or other transfers of value made or distributed to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, the physicians and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members. Beginning in 2022, applicable manufacturers also will be required to report such information regarding its payments and other transfers of value to physician assistants, nurse practitioners, clinical nurse specialists, anesthesiologist assistants, certified registered nurse anesthetists and certified nurse midwives during the previous year. In addition, many states also govern the reporting of payments or other transfers of value, many of which differ from each other in significant ways, are often not pre-empted, and may have a more prohibitive effect than the Sunshine Act, thus further complicating compliance efforts.

In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of drug and biological products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical and biotechnology companies to establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical and biotechnology companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

Ensuring business arrangements with third parties comply with applicable healthcare laws and regulations is a costly endeavor. If our operations are found to be in violation of any of the federal and state healthcare laws described above or any other current or future governmental regulations that apply to us, we may be subject to significant penalties, including without limitation, civil, criminal and/or administrative penalties, damages, fines, disgorgement, individual imprisonment, exclusion from participation in government programs, such as Medicare and Medicaid, injunctions, private "qui tam" actions brought by individual whistleblowers in the name of the government, or refusal to allow us to enter into government contracts, contractual damages, reputational harm, administrative burdens, diminished profits and future earnings, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we may obtain regulatory approval. In the United States and in foreign markets, sales of any products for which we receive regulatory approval for commercial sale will depend, in part, on the extent to which third-party payors provide coverage and establish adequate reimbursement levels for such products. In the United States, third-party payors include federal and state healthcare programs, private managed care providers, health insurers and other organizations. Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid in the United States, and commercial payors are critical to new product acceptance.

Third-party payors decide which therapeutics they will pay for and establish reimbursement levels. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a therapeutic is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

We cannot be sure that reimbursement will be available for any product that we commercialize and, if coverage and reimbursement are available, we cannot be sure that the level of reimbursement will be adequate. Coverage may also be more limited than the purposes for which the product is approved by the FDA or comparable foreign regulatory authorities. Limited coverage and less than adequate reimbursement may reduce the demand for, or the price of, any product for which we obtain regulatory approval.

Third-party payors are increasingly challenging the price, examining the medical necessity, and reviewing the cost-effectiveness of medical products, therapies and services, in addition to questioning their safety and efficacy. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with branded drugs and drugs administered under the supervision of a physician. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA approvals. Our product candidates may not be considered medically necessary or cost-effective. Obtaining coverage and reimbursement approval of a product from a third-party payor is a time-consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost-effectiveness data for the use of our product on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. A third-party payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Additionally, in the United States there is no uniform policy among third-party payors for coverage or reimbursement. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies, but also have their own methods and approval processes. Therefore, one third-party payor's determination to provide coverage for a product does not assure that other payors will also provide coverage for the product. Adequate third-party payor reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize any product candidate that we successfully develop.

Certain of our products, once approved, may be administered by a physician. Under currently applicable U.S. law, certain products not usually self-administered (including injectable drugs) may be eligible for coverage under Medicare through Medicare Part B. Medicare Part B is part of original Medicare, the federal health care program that provides health care benefits to the aged and disabled, and covers outpatient services and supplies, including certain pharmaceutical products, that are medically necessary to treat a beneficiary's health condition. As a condition of receiving Medicare Part B reimbursement for a manufacturer's eligible drugs or biologicals, the manufacturer is required to participate in other government healthcare programs, including the Medicaid Drug Rebate Program and the 340B Drug Pricing Program. The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the HHS as a

condition for states to receive federal matching funds for the manufacturer's outpatient drugs furnished to Medicaid patients. Under the 340B Drug Pricing Program, the manufacturer must extend discounts to entities that participate in the program.

Different pricing and reimbursement schemes exist in other countries. In the European Union, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines but monitor and control company profits. The downward pressure on health care costs has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any product candidates for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide coverage and adequate reimbursement. In addition, emphasis on managed care, the increasing influence of health maintenance organizations, and additional legislative changes in the United States has increased, and we expect will continue to increase, the pressure on healthcare pricing. The downward pressure on the rise in healthcare costs in general, particularly prescription medicines, medical devices and surgical procedures and other treatments, has become very intense. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Healthcare Reform

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of product candidates, restrict or regulate post-approval activities, and affect the ability to profitably sell product candidates for which marketing approval is obtained. Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives.

For example, the ACA has substantially changed healthcare financing and delivery by both governmental and private insurers. Among the ACA provisions of importance to the pharmaceutical and biotechnology industries, in addition to those otherwise described above, are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain specified branded prescription drugs and biologic agents apportioned among these entities according to their market share in some government healthcare programs that began in 2011;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program, retroactive to January 1, 2010, to 23.1% and 13% of the average manufacturer
- price for most branded and generic drugs, respectively, and capped the total rebate amount for innovator drugs at 100% of the average manufacturer price;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% (and 70% starting on January 1, 2019) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturers' outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for individuals with income at or below 133% of the federal poverty level, thereby potentially increasing manufacturers' Medicaid rebate liability;
- expansion of the entities eligible for discounts under the 340B Drug Discount Program;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research;
- expansion of healthcare fraud and abuse laws, including the FCA and the federal Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted, or injected;
- requirements to report certain financial arrangements with physicians and teaching hospitals;
- a requirement to annually report certain information regarding drug samples that manufacturers and distributors provide to physicians;
- establishment of a Center for Medicare Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending; and
- a licensure framework for follow on biologic products.

There have been legal and political challenges to certain aspects of the ACA. Since January 2017, President Trump signed several executive orders and other directives designed to delay, circumvent, or loosen certain requirements mandated by the ACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, several bills affecting the implementation of certain taxes under the ACA have been signed into law. In December 2017, the Tax Cuts and Jobs Act of 2017 was enacted which repeals, effective January 1, 2019, the tax penalty for an individual's failure to maintain ACA-mandated health insurance, commonly referred to as the "individual mandate." Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the Affordable Care Act's mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminated the health insurer tax. Further, the Bipartisan Budget Act of 2018, or the BBA, among other things, amends the ACA, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole."

On December 14, 2018, a Texas U.S. District Court Judge ruled that the Affordable Care Act is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the Affordable Care Act are invalid as well. The U.S. Supreme Court is currently reviewing this case, but it is unknown when a decision will be reached. Although the U.S. Supreme Court has yet ruled on the constitutionality of the ACA, on January 28, 2021, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021 through May 15, 2021 for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructs certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. It is unclear how the Supreme Court ruling, other such litigation, and the healthcare reform measures of the Biden administration will impact the ACA and our business.

Other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect beginning on April 1, 2013 and, due to subsequent legislative amendments to the statute, including the BBA, will stay in effect through

2030 unless additional Congressional action is taken. These reductions have been suspended from May 1, 2020 through March 31, 2021 due to the COVID-19 pandemic. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Further, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. At the federal level, the Trump administration used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives. For example, on July 24, 2020 and September 13, 2020, President Trump announced several executive orders related to prescription drug pricing that attempt to implement several of the Trump administration's proposals. The FDA also released a final rule, effective November 30, 2020, implementing a portion of the importation executive order providing guidance for states to build and submit importation plans for drugs from Canada. Further, on November 20, 2020, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The implementation of the rule has been delayed by the Biden administration from January 1, 2022 to January 1, 2023 in response to ongoing litigation. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a new safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers, the implementation of which have also been delayed pending review by the Biden administration until March 22, 2021. On November 20, 2020, CMS issued an interim final rule implementing President Trump's Most Favored Nation executive order, which would tie Medicare Part B payments for certain physician-administered drugs to the lowest price paid in other economically advanced countries, effective January 1, 2021. On December 28, 2020, the United States District Court in Northern California issued a nationwide preliminary injunction against implementation of the interim final rule. However, it is unclear whether the Biden administration will work to reverse these measures or pursue similar policy initiatives. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures.

Further, it is possible that additional governmental action will be taken in response to the COVID-19 pandemic.

The Foreign Corrupt Practices Act

The Foreign Corrupt Practices Act, or the FCPA, prohibits any U.S. individual or business from paying, offering, or authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring us to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Additional Regulation

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations.

Other Regulations

We are also subject to numerous federal, state and local laws relating to such matters as safe working conditions, manufacturing practices, environmental protection, fire hazard control, and disposal of hazardous or potentially hazardous substances. We may incur significant costs to comply with such laws and regulations now or in the future.

Employees

As of December 31, 2020, we had 206 employees, 110 of whom hold advanced degrees, including 54 with a Ph.D. and/or M.D. degree. Of these employees, 168 were engaged in research and development activities and 38 were engaged in general and administrative activities. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Human Capital Resources

We have grown to a team of 206 employees as of December 31, 2020, all of whom were employed in the United States. Our highly qualified and experienced team which includes scientists, physicians and professionals across research, clinical, manufacturing, regulatory, and general & administrative functions are critical to our success. We also leverage temporary workers to provide flexibility for our business needs. During 2020, we added over 80 new employees to our team.

We expect to continue to add additional employees in 2021 with a focus on expanding our expertise and capabilities in clinical and preclinical research and development, including an expansion of our internal manufacturing capacity. Our culture is driven by innovation, nimbleness and passion for the work that we do, the people we work with and the patients we serve. As we grow, we continually evaluate our business needs and opportunities and balance hiring top talent internally and leveraging external expertise. Currently, we remain reliant on third-party contract manufacturers and clinical research organizations for our clinical programs.

Corporate Information

We were incorporated in Delaware in December 2014. Our principal executive offices are located at 9390 Towne Centre Drive, Suite 200, San Diego, California 92121, and our telephone number is (858) 779-3100. Our corporate website address is www.poseida.com. Information contained on or accessible through our website is not a part of this Annual Report, and the inclusion of our website address in this report is an inactive textual reference only.

Emerging Growth Company

We are an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act of 2012. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of our initial public offering, or IPO, in July 2020, (b) in which we have total annual gross revenue of at least \$1.07 billion, or (c) in which we are deemed to be a large accelerated filer, which means we have been subject to the reporting requirements of the Exchange Act for twelve calendar months and the market value of our common stock that is held by non-affiliates exceeded \$700.0 million as of the prior June 30th, and (2) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period. We refer to the Jumpstart Our Business Startups Act of 2012 in this Annual Report as the “JOBS Act,” and references to “emerging growth company” have the meaning associated with it in the JOBS Act.

Item 1A. Risk Factors.

An investment in our common stock is speculative and involves a high degree of risk. You should consider carefully the risks described below, together with the other information contained in this Annual Report on Form 10-K, including our consolidated financial statements and the related notes and in the section titled “Management’s

Discussion and Analysis of Financial Condition and Results of Operations” before deciding whether to purchase, hold or sell shares of our common stock. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment. This Annual Report on Form 10-K also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements as a result of a number of factors, including the risks described below. See the section titled “Forward-Looking Statements.”

Summary of Risks Associated with Our Business

Below is a summary of the principal factors that make an investment in our securities speculative or risky. This summary does not address all of the risks that we face. Additional discussion of the risks summarized in this risk factor summary, and other risks that we face, can be found below this risk factor summary and should be carefully considered.

- The COVID-19 pandemic continues to adversely impact our business, including our clinical trials, supply chain and business development activities.
- We are a clinical-stage biopharmaceutical company with a limited operating history. We have incurred net losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future. We have never generated any revenue from product sales and may never be profitable.
- Our product candidates are in the early stages of development and we have a limited history of conducting clinical trials to test our product candidates in humans. We may not be able to successfully complete clinical development of any of our product candidates.
- Our product candidates are based on novel technologies, which make it difficult to predict the timing, results and cost of product candidate development and likelihood of obtaining regulatory approval.
- Serious adverse events, undesirable side effects or other unexpected properties of our product candidates may be identified during development or after approval, which could lead to the discontinuation of our clinical development programs, refusal by regulatory authorities to approve our product candidates or, if discovered following marketing approval, revocation of marketing authorizations or limitations on the use of our product candidates.
- We rely on third parties to conduct our clinical trials, perform some of our research and preclinical studies and manufacture and supply certain of our product candidates. If these third parties do not satisfactorily carry out their contractual duties or fail to meet expected deadlines, our development programs may be delayed or subject to increased costs.
- We face substantial competition, which may result in others discovering, developing or commercializing products more quickly or marketing them more successfully than us.
- We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.
- We are currently party to several in-license agreements under which we acquired rights to use, develop, manufacture and/or commercialize certain of our gene engineering technologies and resulting product candidates. If we breach our obligations under these agreements, we may be required to pay damages, lose our rights to these technologies or both.
- We will need to obtain substantial additional funding to complete the development and any commercialization of our product candidates. If we are unable to raise this capital when needed, we may be forced to delay, reduce or eliminate our product development programs or other operations.
- If we are unable to obtain and maintain sufficient intellectual property protection for our platform technologies and product candidates, or if the scope of the intellectual property protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours.

- If we are sued for infringing intellectual property rights of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing our product candidates.

Risks Related to Our Limited Operating History, Financial Position and Capital Requirements

We are a clinical-stage biopharmaceutical company with a limited operating history. We have incurred net losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future. We have never generated any revenue from product sales and may never be profitable.

We are a clinical-stage biopharmaceutical company with a limited operating history that may make it difficult to evaluate the success of our business to date and to assess our future viability. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, establishing and protecting our intellectual property portfolio, developing our platform technologies, identifying potential product candidates and undertaking research and development and manufacturing activities, including preclinical studies and clinical trials of our product candidates. All of our product candidates are in early development, and none have been approved for commercial sale. We have never generated any revenue from product sales and have incurred net losses each year since we commenced operations. For the years ended December 31, 2020 and 2019 our net losses were \$129.8 million and \$86.5 million, respectively. As of December 31, 2020, we had an accumulated deficit of \$281.9 million. We expect that it will be several years, if ever, before we have a product candidate ready for regulatory approval and commercialization. We expect to incur increasing levels of operating losses over the next several years and for the foreseeable future as we advance our product candidates through clinical development. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital.

To become and remain profitable, we must develop and eventually commercialize a product or products with significant market potential. This will require us to be successful in a range of challenging activities, including completing preclinical studies and clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we may obtain marketing approval and satisfying any post-marketing requirements. We may never succeed in these activities and, even if we succeed in commercializing one or more of our product candidates, we may never generate revenue that is significant or large enough to achieve profitability. In addition, as a young business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown challenges. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis and we will continue to incur substantial research and development and other expenditures to develop and market additional product candidates. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We will need to obtain substantial additional funding to complete the development and any commercialization of our product candidates. If we are unable to raise this capital when needed, we may be forced to delay, reduce or eliminate our product development programs or other operations.

Since our inception, we have used substantial amounts of cash to fund our operations and expect our expenses to increase substantially during the next few years. The development of biopharmaceutical product candidates is capital intensive. As our product candidates enter and advance through preclinical studies and clinical trials, we will need substantial additional funds to expand our clinical, regulatory, quality and manufacturing capabilities. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to marketing, sales, manufacturing and distribution.

As of December 31, 2020, we had \$309.2 million in cash, cash equivalents and short-term investments. Based upon our current operating plan, we believe that our existing cash, cash equivalents and short-term investments will enable us to fund our operations through at least the next 12 months. However, our current cash, cash equivalents and short-term investments will not be sufficient to fund any of our product candidates through regulatory approval, and we will need to raise substantial additional capital to complete the development and commercialization of our product candidates.

We have based these estimates on assumptions that may prove to be incorrect or require adjustment as a result of business decisions, and we could exhaust our available capital resources sooner than we currently expect. Our future capital requirements will depend on many factors, including:

- scope, progress and results of our ongoing and planned preclinical studies and clinical trials for our product candidates;
- unanticipated serious safety concerns related to the use of our product candidates;
- timing of licensing payments we may be required to make based on the development of our product candidates;
- the number, and development requirements of other product candidates that we may pursue;
- the timing and outcome of regulatory review of our product candidates;
- changes in laws or regulations applicable to our product candidates, including but not limited to clinical trial requirements for approval;
- our decisions to initiate additional clinical trials, not to initiate any clinical trial or to terminate an existing clinical trial;
- the cost of obtaining raw materials and drug product for clinical trials and commercial supply, which, due to the wide variability in manufacturing costs between autologous and allogeneic product candidates, will also depend on which product candidates progress to future clinical trials;
- whether we decide to partner any of our product candidates with any third parties and the terms of any such partnership or collaboration;
- the cost and timing of establishing and validating our pilot manufacturing facility;
- whether we decide to establish a commercial manufacturing facility for supply of our product candidates; and
- additions or departures of key scientific or management personnel.

Because we do not expect to generate revenue from product sales for many years, if at all, we will need to obtain substantial additional funding in connection with our continuing operations and expected increases in expenses. Until such time as we can generate significant revenue from sales of our product candidates, if ever, we expect to finance our cash needs through equity offerings, debt financings or other capital sources, including potentially grants, collaborations, licenses or other similar arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. The impact of the COVID-19 pandemic on capital markets may affect the availability, amount and type of financing available to us in the future. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

The terms of our loan agreement place restrictions on our operating and financial flexibility. If we raise additional capital through debt financing, the terms of any new debt could further restrict our ability to operate our business.

We have an outstanding term loan in the principal amount of \$30.0 million under our loan and security agreement, as amended, with Oxford Finance LLC, or Oxford. The loan is secured by a lien covering substantially all of our personal property, rights and assets, excluding intellectual property. The loan agreement contains customary affirmative and negative covenants and events of default applicable to us and any subsidiaries. The affirmative covenants include, among others, covenants requiring us (and us to cause our subsidiaries, if any) to maintain governmental approvals, deliver certain financial reports, maintain insurance coverage, keep inventory, if any, in good and marketable condition and protect material intellectual property. The negative covenants include, among others, restrictions on us and our subsidiaries transferring collateral, incurring additional indebtedness, engaging in mergers or acquisitions, paying cash dividends or making other distributions, making investments, creating liens, selling assets and making any payment on subordinated debt, in each case subject to certain

exceptions. The restrictive covenants of the loan agreement could cause us to be unable to pursue business opportunities that we or our stockholders may consider beneficial. In addition, Oxford could declare a default upon the occurrence of any event that it interprets as a material adverse change as defined under the loan agreement. If we default under the loan agreement, Oxford may accelerate all of our repayment obligations and take control of our pledged assets, potentially requiring us to renegotiate our agreement on terms less favorable to us or to immediately cease operations. Further, if we are liquidated, Oxford's right to repayment would be senior to the rights of the holders of our common stock to receive any proceeds from the liquidation. Any declaration by Oxford of an event of default could significantly harm our business and prospects and could cause the price of our common stock to decline. If we raise any additional debt financing, the terms of such additional debt could further restrict our operating and financial flexibility.

Risks Related to the Discovery, Development and Regulatory Approval of Our Product Candidates

Our product candidates are in the early stages of development and we have a limited history of conducting clinical trials to test our product candidates in humans.

We are early in our development efforts and most of our operations to date have been limited to developing our platform technologies, establishing manufacturing capabilities and conducting drug discovery and preclinical studies. Our lead product candidate, P-BCMA-101, entered a Phase 1 clinical trial in December 2017, which was the first time one of our product candidates had been tested in humans, and we dosed the first patient in a Phase 1 clinical trial of our second product candidate, P-PSMA-101, in May 2020. As a result, we have limited infrastructure, experience conducting clinical trials as a company and regulatory interactions, and cannot be certain that our clinical trials will be completed on time, that our planned clinical trials will be initiated on time, if at all, that our planned development programs would be acceptable to the FDA or other comparable foreign regulatory authorities, or that, if approval is obtained, such product candidates can be successfully commercialized.

Because of the early stage of development of our product candidates, our ability to eventually generate significant revenues from product sales will depend on a number of factors, including:

- successful completion of preclinical studies;
- submission of our INDs or other regulatory applications for our planned clinical trials or future clinical trials and authorizations from regulators to initiate clinical studies;
- successful enrollment in, and completion of, clinical trials and achieving positive results from the trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing manufacturing capabilities or arrangements with third-party manufacturers for clinical supply and, if and when approved, for commercial supply;
- establishing sales, marketing and distribution capabilities and launching commercial sales of our products, if and when approved, whether alone or in combination with others;
- acceptance of our products, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- developing and implementing marketing and reimbursement strategies;
- obtaining and maintaining third-party coverage and adequate reimbursement;
- obtaining and maintaining patent, trade secret and other intellectual property protection and regulatory exclusivity for our product candidates;
- the ability to obtain clearance or approval of companion diagnostic tests, if required, on a timely basis, or at all; and
- maintaining a continued acceptable safety profile of any product following approval, if any.

If we do not achieve one or more of these requirements in a timely manner, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business.

Clinical development is a lengthy, expensive and uncertain process. The results of preclinical studies and early clinical trials are not always predictive of future results. Any product candidate that we advance into clinical trials may not achieve favorable results in later clinical trials, if any, or receive marketing approval.

The research and development of drugs and biological products is extremely risky. Only a small percentage of product candidates that enter the development process ever receive marketing approval. Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, can take many years to complete and its outcome is uncertain.

The results of preclinical studies and early clinical trials of our product candidates and other products, even those with the same or similar mechanisms of action, may not be predictive of the results of later-stage clinical trials. In particular, it is not uncommon for product candidates to exhibit unforeseen safety or efficacy issues when tested in humans despite promising results in preclinical animal models. While we have conducted preclinical studies and have interim Phase 1 clinical trial results for P-BCMA-101 at certain dose levels, we do not know how P-BCMA-101 will perform in the ongoing Phase 1 clinical trial or in future clinical trials, whether any initial tumor responses observed to date will be durable or whether adverse events will arise over time. In August 2020, we announced the P-PSMA-101 trial was put on clinical hold to assess a patient death. This clinical hold was lifted in November 2020 with the implementation of protocol amendments intended to increase patient compliance and safety that include modified inclusion and exclusion criteria and frequency of monitoring and laboratory testing. In addition, due primarily to the observation of anti-drug antibodies in some patients, we are currently exploring additional dosing strategies, such as administering the doses in smaller cycles in the first 30 days and adding rituximab to the preconditioning regime to potentially suppress any antibody response, in the expanded Phase 1 portion of our clinical program. If these anti-drug antibodies are neutralizing the product candidate, the activity of P-BCMA-101, or any other product candidate in which anti-drug antibodies neutralize the product candidate, may be limited. To the extent that we choose one of these newer dosing strategies for advancement in our Phase 2 clinical trial, it may be on the basis of more limited data as compared to the previously evaluated Phase 1 cohorts, and therefore we may have limited ability to predict how P-BCMA-101 will perform in the Phase 2 clinical trial. Further, the P-BCMA-101 product used in our ongoing exploratory dosing cohorts was manufactured at a different facility using revised methods from those used in our original Phase 1 cohorts, which could adversely affect the results of such cohorts and future trials. Similarly, for P-PSMA-101, we dosed the first patient in a Phase 1 clinical trial in May 2020. Other than P-BCMA-101 and P-PSMA-101, none of our product candidates have ever been tested in humans. Future results of preclinical and clinical testing of our product candidates are also less certain due to the novel and relatively untested nature of our approach to CAR-T and gene therapy development and related platform technologies. In general, clinical trial failure may result from a multitude of factors including flaws in study design, dose selection, patient enrollment criteria and failure to demonstrate favorable safety or efficacy traits. As such, failure in clinical trials can occur at any stage of testing. A number of companies in the biopharmaceutical industry have suffered setbacks in the advancement of clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials.

If the results of our clinical trials are inconclusive or if there are safety concerns or adverse events associated with our product candidates, we may:

- incur unplanned costs;
- be delayed in or prevented from obtaining marketing approval for our product candidates;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings including boxed warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the product or impose restrictions on its distribution in the form of a modified Risk Evaluation and Mitigation Strategy, or REMS;

- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Treatment with our oncology product candidates involves chemotherapy and myeloablative treatments, which can cause side effects or adverse events that are unrelated to our product candidate but may still impact the success of our clinical trials. Additionally, our product candidates could potentially cause other adverse events. The inclusion of critically ill patients in our clinical trials may result in deaths or other adverse medical events due to other therapies or medications that such patients may be using. As described above, any of these events could prevent us from obtaining regulatory approval or achieving or maintaining market acceptance of our product candidates and impair our ability to commercialize our products. Because all of our product candidates are derived from our platform technologies, a clinical failure of one of our product candidates may also increase the actual or perceived likelihood that our other product candidates will experience similar failures.

We may encounter substantial delays in our clinical trials.

We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. For example, we cannot begin our planned Phase 1 clinical trials for P-BCMA-ALLO1 or P-MUC1C-ALLO1, our dual CAR product candidates or our liver directed gene therapy candidates until we complete certain preclinical development and submit and receive authorization to proceed under INDs. In addition, we initiated a Phase 2 clinical trial for P-BCMA-101 while our Phase 1 clinical trial continues, and we cannot predict whether results observed in the ongoing Phase 1 clinical trial, including from new dosing levels, strategies or schedules or manufacturing changes, will delay the completion of the Phase 2 clinical trial. We announced in August 2020 our P-PSMA-101 trial was put on clinical hold to assess a patient death. In November 2020 we announced that the FDA had lifted the clinical hold based upon our investigation of the event and proposed protocol amendments intended to increase patient compliance and safety. While we have resumed the trial, the clinical hold will delay the ultimate completion of the trial and we cannot guarantee that after resuming the trial, we will not observe additional patient deaths or encounter other events that cause the P-PSMA-101 trial be suspended or terminated. Finally, the COVID-19 pandemic has impacted clinical trials broadly, including our own, with some sites pausing enrollment and we have experienced a delay in manufacturing at times due to potential exposure. These impacts have caused us to reevaluate the expected timing of clinical milestones and we have and continue to experience delays in site initiation and patient enrollment, and could also experience delays in the manufacture of our product candidates for clinical testing and other difficulties in starting or competing our clinical trials. Other events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory agencies on trial design;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- delays in obtaining required institutional review board, or IRB, approval at each clinical trial site;
- delays in recruiting suitable patients to participate in our clinical trials;
- imposition of a clinical hold by regulatory agencies, after an inspection of our clinical trial operations or study sites;
- failure by our CROs, other third parties or us to adhere to the trial protocol or the FDA's good clinical practices, or GCPs, or applicable regulatory guidelines in other countries;
- third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other comparable foreign regulatory authorities for violations of applicable regulatory requirements;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the treatment sites, including due to a facility manufacturing any of our product candidates or any of their components being ordered by the FDA or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of current good manufacturing practices, or cGMPs, regulations or other applicable requirements, or infections or cross-contaminations of product candidates in the manufacturing process;

- delays in having patients complete participation in a study or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a study;
- discovering that product candidates have unforeseen safety issues, undesirable side effects or other unexpected characteristics;
- to the extent that we conduct clinical trials in foreign countries, the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries;
- receiving untimely or unfavorable feedback from applicable regulatory authorities regarding the trial or requests from regulatory authorities to modify the design of a trial;
- suspensions or terminations by us, the IRBs of the institutions at which such trials are being conducted, by the Data Safety Monitoring Board, for such trial or by regulatory authorities due to a number of factors, including those described above;
- lack of adequate funding; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to raise capital, generate revenues from product sales and enter into or maintain collaboration arrangements. For example, under certain of our manufacturing agreements for our product candidates we pay a fixed price per month for up to a specified number of manufacturing runs and certain clinical trial services agreements are based on fees that do not vary based on patient enrollment. Therefore, if enrollment in a clinical trial is slowed, certain of our expenses related to the trial would not decrease and therefore the overall costs to complete the trial would increase. In addition, if we make manufacturing changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

Our product candidates are based on novel technologies, which make it difficult to predict the timing, results and cost of product candidate development and likelihood of obtaining regulatory approval.

We have concentrated our research and development efforts on product candidates using our platform technologies, and our future success depends on the successful development of this approach. CAR-T and gene editing in general are newly-emerging fields and our approaches in particular have not been extensively tested over any significant period of time. In particular, while we believe that CAR-T products with higher percentages of T_{SCM} cells may be capable of overcoming certain challenges faced by early-generation CAR-T products, we cannot be certain that increasing the percentage of these cells will result in the intended benefits or will not result in unforeseen negative consequences over time, including due to the potential long-term persistence of the modified cells in the body. We have not yet succeeded and may not succeed in demonstrating efficacy and safety for any product candidates based on our platform technologies in clinical trials or in obtaining marketing approval thereafter, and use of our platform technologies may not ever result in marketable products. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners or establishing our own commercial manufacturing capabilities, which may prevent us from completing our clinical trials or commercializing any products on a timely or profitable basis, if at all.

In addition, the clinical trial requirements of the FDA, the European Medicines Agency, or EMA, and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. While CAR-T and gene therapy products have made progress in recent years, only a small number of products have been approved in

the United States or other markets, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates.

In addition, the gene-editing industry is rapidly developing, and our competitors may introduce new technologies that render our technologies obsolete or less attractive. New technology could emerge at any point in the development cycle of our product candidates. As competitors use or develop alternative technologies, any failures of such technologies could adversely impact our programs. For example, some studies have suggested that gene editing using the CRISPR-Cas9 method may increase the risk that the edited cells themselves become cancerous. Regardless of our belief that our non-viral Cas-CLOVER approach to gene editing may avoid some of the issues identified in these studies, it is possible that our approach will be associated with similar risks or that issues encountered with other gene editing techniques will create a negative perception of or increase scrutiny for our technologies and product candidates.

Regulatory requirements governing products created with gene editing technology or involving gene therapy treatment have changed frequently and will likely continue to change in the future. Approvals by one regulatory agency may not be indicative of what any other regulatory agency may require for approval, and there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of gene therapy products and other products created with gene editing technology. For example, under the National Institutes of Health, or NIH, Guidelines for Research Involving Recombinant DNA Molecules, or NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. Even though we may not be required to submit a protocol for our product candidates through the NIH for review, we will still be subject to significant regulatory oversight by the FDA, and in addition to the government regulators, the applicable IBC and IRB of each institution at which we conduct clinical trials of our product candidates, or a central IRB if appropriate, would need to review and approve the proposed clinical trial.

Additionally, adverse developments in clinical trials conducted by others of gene therapy products or products created using genome editing technology, such as products developed through the application of a CRISPR/Cas9 technology, or adverse public perception of the field of gene editing, may cause the FDA and other regulatory bodies to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing gene editing technologies, either of which could materially harm our business. Furthermore, regulatory action or private litigation could result in expenses, delays or other impediments to our research programs or the development or commercialization of current or future product candidates.

We are also developing allogeneic CAR-T product candidates that are engineered from healthy donor T cells and are intended for use in any patient with certain cancers. Allogeneic versions of CAR-T product candidates is an unproven field of development and is subject to particular risks that are difficult to quantify, including understanding and addressing variability in the quality of a donor's T cells and the patient's potential immune reaction to the foreign donor cells, which could ultimately affect safety, efficacy and our ability to produce product in a reliable and consistent manner.

Serious adverse events, undesirable side effects or other unexpected properties of our product candidates may be identified during development or after approval, which could lead to the discontinuation of our clinical development programs, refusal by regulatory authorities to approve our product candidates or, if discovered following marketing approval, revocation of marketing authorizations or limitations on the use of our product candidates thereby limiting the commercial potential of such product candidate.

To date, we have only tested P-BCMA-101 in a limited number of patients with cancer and the majority of these clinical trial participants have only been observed for a limited period of time after dosing. As we continue developing our product candidates and initiate clinical trials of our additional product candidates, serious adverse events, or SAEs, undesirable side effects, relapse of disease or unexpected characteristics may emerge causing us to

abandon these product candidates or limit their development to more narrow uses or subpopulations in which the SAEs or undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective or in which efficacy is more pronounced or durable. For example, a significant risk observed in CAR-T product clinical trials is the development of CRS which in some instances resulted in neurotoxicity and patient deaths. In our ongoing Phase 1 clinical trial of P-BCMA-101, as of the data cutoff of November 16, 2020, the rate of CRS remained low at approximately 20%, with none being \geq Grade 3. There were also two patients reported to have neurotoxicity related to P-BCMA-101, one case (confusion) observed in a patient with mental status changes before administration of P-BCMA-101 and another case (aphasia) was reported in a patient in conjunction with pneumonia and sepsis, and no deaths related to P-BCMA-101. Overall other common treatment emergent adverse events and SAEs have included cytopenias, infections and constitutional symptoms, which are mostly consistent with conditioning lymphodepletion therapy and the underlying disease and not generally believed by us to be related to CAR-T therapies like P-BCMA-101. Should we observe additional or more severe cases of CRS in our clinical trials or identify other undesirable side effects or other unexpected findings depending on their severity, our trials could be delayed or even stopped and our development programs may be halted entirely. In August 2020, we announced our P-PSMA-101 trial was placed on clinical hold to evaluate the death of a patient, which may have been related to treatment with P-PSMA-101. In November 2020 we announced that the FDA had lifted the clinical hold based upon our investigation of the event and proposed protocol amendments intended to increase patient compliance and safety, and we have resumed the trial. Despite the clinical hold being lifted, we could observe similar patient deaths or other adverse events that require that the P-PSMA-101 trial be suspended or terminated, which could represent a substantial setback to the program.

Even if our product candidates initially show promise in early clinical trials, the side effects of biological products are frequently only detectable after they are tested in larger, longer and more extensive clinical trials or, in some cases, after they are made available to patients on a commercial scale after approval. Sometimes, it can be difficult to determine if the serious adverse or unexpected side effects were caused by the product candidate or another factor, especially in oncology subjects who may suffer from other medical conditions and be taking other medications. If serious adverse or unexpected side effects are identified during development or after approval and are determined to be attributed to our product candidate, we may be required to develop a REMS to ensure that the benefits of treatment with such product candidate outweigh the risks for each potential patient, which may include, among other things, a communication plan to health care practitioners, patient education, extensive patient monitoring or distribution systems and processes that are highly controlled, restrictive and more costly than what is typical for the industry. Product-related side effects could also result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

In addition, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may suspend, withdraw or limit approvals of such product, or seek an injunction against its manufacture or distribution;
- regulatory authorities may require additional warnings on the label, including “boxed” warnings, or issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way a product is administered or conduct additional clinical trials;
- the product may become less competitive;
- we may decide to remove the product from the marketplace; and
- we may be subject to fines, injunctions or the imposition of civil or criminal penalties.

Interim, topline and preliminary data from our clinical trials may change as more patient data become available, and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose preliminary, interim or topline data from our preclinical studies and clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change as patient enrollment and treatment continues and more patient data become available. Adverse differences between previous preliminary or interim data and future interim or final data could significantly harm our business prospects. We may also announce topline data following the completion of a preclinical study or clinical trial, which may be subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the interim, topline or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim, topline and preliminary data should be viewed with caution until the final data are available.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine to be material or otherwise appropriate information to include in our disclosure.

We may not ultimately realize the potential benefits of orphan drug designation for P-BCMA-101.

We received orphan drug designation for P-BCMA-101 for the treatment of relapsed/refractory multiple myeloma. The FDA grants orphan designation to drugs that are intended to treat rare diseases with fewer than 200,000 patients in the United States or that affect more than 200,000 persons but where there is no reasonable expectation to recover the costs of developing and marketing a treatment drug in the United States. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and application fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. However, orphan drug designation neither shortens the development time nor regulatory review time of a product candidate nor gives the candidate any advantage in the regulatory review or approval process.

In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity for the orphan patient population. Exclusive marketing rights in the United States may also be unavailable if we or our collaborators seek approval for an indication broader than the orphan designated indication and may be lost if the FDA later determines that the request for designation was materially defective. Even if we obtain orphan drug designation, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition.

We have received Regenerative Medicine Advanced Therapy, or RMAT, designation by the FDA for P-BCMA-101 for the treatment of multiple myeloma, and may seek RMAT designation for our other product candidates; however, such designations may not lead to a faster development or regulatory review or approval process and do not increase the likelihood that our product candidates will receive marketing approval.

In November 2018, we received RMAT designation for P-BCMA-101 for the treatment of multiple myeloma. In 2017, the FDA established the RMAT designation as part of its implementation of the 21st Century Cures Act.

An investigational drug is eligible for RMAT designation if: (1) it meets the definition of a regenerative medicine therapy, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions; (2) it is intended to treat, modify, reverse, or cure a serious disease or condition; and (3) preliminary clinical evidence indicates that the investigational drug has the potential to address unmet medical needs for such disease or condition. RMAT designation provides potential benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate, and eligibility for rolling review of BLAs and priority review. Product candidates granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion of clinical trials, as appropriate. RMAT-designated product candidates that receive accelerated approval may, as determined by the FDA, fulfill their post-approval requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real-world evidence (such as electronic health records), through the collection of larger confirmatory data sets, or via post-approval monitoring of all patients treated with such therapy prior to approval of the therapy.

RMAT designation does not change the standards for product approval, and there is no assurance that such designation or eligibility for such designation will result in expedited review or approval or that the approved indication will not be narrower than the indication covered by the RMAT designation. Additionally, RMAT designation can be revoked if the criteria for eligibility cease to be met as clinical data emerges.

Our product candidates must meet extensive regulatory requirements before they can be commercialized and any regulatory approval may contain limitations or conditions that require substantial additional development expenses or limit our ability to successfully commercialize the product.

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, import, export, marketing and distribution of our product candidates are subject to extensive regulation by the FDA in the United States and by comparable foreign regulatory authorities in foreign markets. In the United States, we are not permitted to market our product candidates until we receive regulatory approval from the FDA. The process of obtaining regulatory approval is expensive, often takes many years following the commencement of clinical trials and can vary substantially based upon the type, complexity and novelty of the product candidates involved, as well as the target indications and patient population. Despite the time and expense invested in clinical development of product candidates, regulatory approval is never guaranteed.

To date, we have not submitted a BLA or other marketing authorization application to the FDA or similar drug approval submissions to comparable foreign regulatory authorities for any product candidate. With respect to P-BCMA-101, we currently intend to seek accelerated approval following the completion of our Phase 2 clinical trial. However, it is possible that at the time of a BLA submission, P-BCMA-101 would not be eligible for accelerated approval or the FDA could determine that accelerated approval is not warranted. Accelerated approval requires the data to indicate the drug candidate has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or an effect on a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. In particular, because the FDA has already approved therapies for multiple myeloma, and because additional drugs may be approved for multiple myeloma while we are developing P-BCMA-101, it is difficult to predict whether accelerated approval will be possible for P-BCMA-101 at the time we expect to submit a BLA. The FDA has indicated that if data from our Phase 2 clinical trial do not provide evidence sufficient for accelerated approval, that additional clinical testing would be required to support approval, with a preference for us to conduct a randomized controlled trial or trials. While we intend to initiate a randomized Phase 3 clinical trial for P-BCMA-101 following the enrollment of the Phase 2 clinical trial regardless, if we were unable to obtain accelerated approval based on the results of our Phase 2 clinical trial, it would represent a significant delay in the approval of, and our ability to commercialize, P-BCMA-101.

Prior to obtaining approval to commercialize a product candidate in the United States or abroad, we or our potential future collaborators must demonstrate with substantial evidence from adequate and well-controlled clinical trials, and to the satisfaction of the FDA or comparable foreign regulatory authorities, that such product candidates are safe and effective for their intended uses. Even if we believe the preclinical or clinical data for our product

candidates are promising, such data may not be sufficient to support approval by the FDA and comparable foreign regulatory authorities. In particular, because we are seeking to identify and develop product candidates using new technologies, there is heightened risk that the FDA or other regulatory authorities may impose additional requirements prior to granting marketing approval, including enhanced safety studies or monitoring. Furthermore, as more product candidates within a particular class of products proceed through clinical development to regulatory review and approval, the amount and type of clinical data that may be required by regulatory authorities may increase or change.

The FDA or comparable foreign regulatory authorities can delay, limit or deny approval of a product candidate for many reasons, including:

- such authorities may disagree with the design or implementation of our clinical trials;
- negative or ambiguous results from our clinical trials or results may not meet the level of statistical significance required by the FDA or comparable foreign regulatory agencies for approval;
- serious and unexpected product-related side effects may be experienced by participants in our clinical trials or by individuals using biological products similar to our product candidates;
- the population studied in the clinical trial may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- such authorities may not accept clinical data from trials which are conducted at clinical facilities or in countries where the standard of care is potentially different from that of the United States;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- such authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- such authorities may not agree that the data collected from clinical trials of our product candidates are acceptable or sufficient to support the submission of an application for regulatory approval or other submissions or to obtain regulatory approval in the United States or elsewhere, including due to clinical trial issues encountered as a result of COVID-19 pandemic, and such authorities may impose requirements for additional preclinical studies or clinical trials;
- such authorities may disagree regarding the formulation, labeling and/or the specifications of our product candidates;
- approval may be granted only for indications that are significantly more limited than what we apply for and/or with other significant restrictions on distribution and use;
- such authorities may fail to approve any required companion diagnostics to be used with our product candidates;
- such authorities may find deficiencies in the manufacturing processes or facilities of our third-party manufacturers with which we or any of our potential future collaborators contract for clinical and commercial supplies; or
- the approval policies or regulations of such authorities may significantly change in a manner rendering our or any of our potential future collaborators' clinical data insufficient for approval.

With respect to foreign markets, approval procedures vary among countries and, in addition to the foregoing risks, may involve additional product testing, administrative review periods and agreements with pricing authorities. In addition, events raising questions about the safety of certain marketed pharmaceuticals may result in increased cautiousness by the FDA and comparable foreign regulatory authorities in reviewing new products based on safety, efficacy or other regulatory considerations and may result in significant delays in obtaining regulatory approvals.

Even if we eventually complete clinical trials and receive approval to commercialize our product candidates, the FDA or comparable foreign regulatory authority may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, and/or the implementation of a REMS. The FDA or the

comparable foreign regulatory authority also may approve a product candidate for a more limited indication or patient population than we originally requested or may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product. Manufacturers of our products and manufacturers' facilities are also required to comply with cGMP regulations, which include requirements related to quality control and quality assurance, as well as the corresponding maintenance of records and documentation. Further, regulatory authorities must approve these manufacturing facilities before they can be used to manufacture our products, and these facilities are subject to continual review and periodic inspections by the FDA and other comparable foreign regulatory authorities for compliance with cGMP regulations.

Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and would materially and adversely impact our business and prospects.

Even if we receive regulatory approval for any of our product candidates, we will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product candidates, if approved, could be subject to labeling and other restrictions and market withdrawal and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our products.

If the FDA, EMA or any other comparable regulatory authority approves any of our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration requirements and continued compliance with cGMPs and GCP, for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market, or voluntary product recalls;
- fines, untitled or warning letters or holds on clinical trials;
- refusal by the FDA, the EMA or any other comparable regulatory authority to approve pending applications or supplements to approved applications filed by us, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; and
- injunctions or the imposition of civil or criminal penalties.

Moreover, if any of our product candidates are approved, our product labeling, advertising and promotion will be subject to regulatory requirements and continuing regulatory review. The FDA strictly regulates the promotional claims that may be made about biopharmaceutical products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our or our collaborators' ability to commercialize our product candidates, and harm our business, financial condition and results of operations.

In addition, the policies of the FDA, the EMA and other comparable regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the Trump administration may impact our business and industry. Namely, the Trump administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine oversight activities such as implementing statutes through rulemaking, issuance of guidance and review and approval of marketing applications. It is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose restrictions on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements, or if we are unable to maintain regulatory compliance, marketing approval that has been obtained may be lost and we may not achieve or sustain profitability.

Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, or approved or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new biologics to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

Separately, in response to the global COVID-19 pandemic, in March 2020 the FDA announced its intention to postpone most foreign inspections of manufacturing facilities and products and temporarily postpone routine surveillance inspections of domestic manufacturing facilities. Domestic inspections were restarted by FDA on a risk-based basis in July 2020. Regulatory authorities outside the United States may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we must prioritize our research programs and will need to focus our discovery and development on select product candidates and indications. Correctly prioritizing our research and development activities is particularly important for us due to the breadth of potential product candidates and indications that we believe could be pursued using our platform technologies. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may also relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

We may not be successful in our efforts to identify or discover additional product candidates in the future.

Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development for a number of reasons, including:

- our inability to design such product candidates with the properties that we desire; or
- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance.

Research programs to identify new product candidates require substantial technical, financial and human resources. If we are unable to identify suitable additional candidates for preclinical and clinical development, our opportunities to successfully develop and commercialize therapeutic products will be limited.

Risks Related to Manufacturing, Commercialization and Reliance on Third Parties

We rely on third parties to conduct our clinical trials and perform some of our research and preclinical studies. If these third parties do not satisfactorily carry out their contractual duties or fail to meet expected deadlines, our development programs may be delayed or subject to increased costs, each of which may have an adverse effect on our business and prospects.

We do not have the ability to conduct all aspects of our preclinical testing or clinical trials ourselves. As a result, we are and expect to remain dependent on third parties to conduct our ongoing Phase 1 and Phase 2 clinical trials and any future clinical trials of our product candidates. Specifically, CROs, clinical investigators, and consultants play a significant role in the conduct of these trials and the subsequent collection and analysis of data. However, we will not be able to control all aspects of their activities. Nevertheless, we are responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on the CROs and other third parties does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GCP requirements, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area, and comparable foreign regulatory authorities for all of our product candidates in clinical development. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, clinical trial investigators and clinical trial sites. If we or any of our CROs or clinical trial sites fail to comply with applicable GCP requirements, the data generated in our clinical trials may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. In addition, our clinical trials must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to stop and/or repeat clinical trials, which would delay the marketing approval process.

There is no guarantee that any such CROs, clinical trial investigators or other third parties on which we rely will devote adequate time and resources to our development activities or perform as contractually required. These risks are heightened as a result of the efforts of government agencies and the CROs themselves to limit the spread of COVID-19, including quarantines and shelter-in-place orders. If any of these third parties fail to meet expected deadlines, adhere to our clinical protocols or meet regulatory requirements, otherwise performs in a substandard manner, or terminates its engagement with us, the timelines for our development programs may be extended or delayed or our development activities may be suspended or terminated. If any of our clinical trial sites terminates for any reason, we may experience the loss of follow-up information on subjects enrolled in such clinical trials unless we are able to transfer those subjects to another qualified clinical trial site, which may be difficult or impossible. In addition, clinical trial investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, or the FDA or any comparable foreign regulatory authority concludes that the financial relationship may have affected the interpretation of the trial, the integrity of the data generated at the applicable clinical trial site may be questioned and the utility of the clinical trial itself may be jeopardized, which could result in the delay or rejection of any marketing application we submit by the FDA or any comparable foreign regulatory authority. Any such delay or rejection could prevent us from commercializing our product candidates.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our products.

We or the third parties on which we rely for the manufacturing and supply of certain of our product candidates for use in preclinical testing and clinical trials, may not be able to establish or maintain supply of our product candidates that is of satisfactory quality and quantity.

We produce in our laboratory relatively small quantities of product for evaluation in our research programs. We rely on third parties for the manufacture of certain of our product candidates for preclinical and clinical testing and may continue to rely on such third parties for commercial manufacture if any of our product candidates are approved. We have a pilot manufacturing facility which we expect to be used to develop and manufacture preclinical and clinical material for future clinical trials for certain product candidates. We expect to use the facility initially for the GMP manufacturing of P-MUC1C-ALLO1 program. We may encounter delays, quality or other issues if and when we begin to use our pilot manufacturing facility for clinical supply. Even after the pilot manufacturing facility is validated and qualified, we expect that we will continue to rely on third parties for various manufacturing needs. We currently have limited manufacturing arrangements and expect that each of our product candidates will only be covered by single source suppliers for the foreseeable future. This reliance increases the risk that we will not have sufficient quantities of our product candidates or products, if approved, or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

Furthermore, all entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in clinical trials must be manufactured in accordance with cGMP requirements. These regulations govern manufacturing processes and procedures, including record keeping, and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA on a timely basis and must adhere to the FDA's Good Laboratory Practice regulations and cGMP regulations enforced by the FDA through its facilities inspection program. Comparable foreign regulatory authorities may require compliance with similar requirements. Our facilities and quality systems, and those of our third-party contract manufacturers, must pass a pre-approval inspection for compliance with the applicable regulations as a condition of marketing approval of our product candidates. We do not control the manufacturing activities of, and are completely dependent on, our contract manufacturers for compliance with cGMP regulations.

In the event that any of our manufacturers fails to comply with such requirements or to perform its obligations to us in relation to quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, including due to the impact of the COVID-19 pandemic, we may be forced to manufacture the materials ourselves, for which we currently do not have the capabilities or resources, or enter into an agreement with another third-party, which we may not be able to do on commercially reasonable terms, if at all. In particular, any replacement of our manufacturers could require significant effort and expertise because there may be a limited number of qualified replacements. In some cases, the technical skills or technology required to manufacture our product candidates may be unique or proprietary to the original manufacturer and we may have difficulty transferring such skills or technology to another third-party and a feasible alternative may not exist. In addition, certain of our product candidates and our own proprietary methods have never been produced or implemented outside of our company, and we may therefore experience delays to our development programs if and when we attempt to establish new third-party manufacturing arrangements for these product candidates or methods. These factors would increase our reliance on such manufacturer or require us to obtain a license from such manufacturer in order to have another third-party manufacture our product candidates. If we are required to or voluntarily change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations and guidelines. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates in a timely manner or within budget.

Our or a third-party's failure to execute on our manufacturing requirements, do so on commercially reasonable terms and comply with cGMP could adversely affect our business in a number of ways, including:

- an inability to initiate or continue clinical trials of our product candidates under development;
- delay in submitting regulatory applications, or receiving marketing approvals, for our product candidates;
- loss of the cooperation of future collaborators;
- subjecting third-party manufacturing facilities or our manufacturing facilities to additional inspections by regulatory authorities;
- requirements to cease development or to recall batches of our product candidates; and
- in the event of approval to market and commercialize our product candidates, an inability to meet commercial demands for our product or any other future product candidates.

Manufacturing gene engineered products is complex and we or our third-party manufacturers may encounter difficulties in production. If we or any of our third-party manufacturers encounter such difficulties, our ability to provide supply of our product candidates for clinical trials or our products for patients, if approved, could be delayed or prevented.

Manufacturing gene engineered products is complex and may require the use of innovative technologies to handle living cells. Manufacturing these products requires facilities specifically designed for and validated for this purpose and sophisticated quality assurance and quality control procedures are necessary. Slight deviations anywhere in the manufacturing process, including filling, labeling, packaging, storage and shipping and quality control and testing, may result in lot failures, product recalls or spoilage. When changes are made to the manufacturing process, we may be required to provide preclinical and clinical data showing the comparable identity, strength, quality, purity or potency of the products before and after such changes. If microbial, viral or other contaminations are discovered at manufacturing facilities, such facilities may need to be closed for an extended period of time to investigate and remedy the contamination, which could delay clinical trials and adversely harm our business. The use of biologically derived ingredients can also lead to allegations of harm, including infections or allergic reactions, or closure of product facilities due to possible contamination.

In addition, there are risks associated with large scale manufacturing for clinical trials or commercial scale including, among others, cost overruns, potential problems with process scale-up, process reproducibility, stability issues, compliance with good manufacturing practices, lot consistency and timely availability of raw materials. Even if we obtain marketing approval for any of our product candidates, there is no assurance that we or our manufacturers will be able to manufacture the approved product to specifications acceptable to the FDA or other comparable foreign regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential commercial launch of the product or to meet potential future demand. If we or our manufacturers are unable to produce sufficient quantities for clinical trials or for commercialization, our development and commercialization efforts would be impaired, which would have an adverse effect on our business, financial condition, results of operations and growth prospects.

Changes in methods of product candidate manufacturing may result in additional costs or delays.

As product candidates progress through preclinical to late-stage clinical trials to marketing approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods, are altered along the way in an effort to optimize yield, manufacturing batch size, minimize costs and achieve consistent quality and results. Such changes carry the risk that they will not achieve these intended objectives. For example, prior to initiating the exploratory cohorts of our ongoing Phase 1 clinical trial, we began manufacturing P-BCMA-101 product at a different facility using revised methods from those used in manufacturing the P-BCMA-101 product that was administered to patients enrolled under the original Phase 1 protocol and for which interim data was previously made available. Any of these changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the altered materials. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or

more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commercialize our product candidates and generate revenue.

Any approved products may fail to achieve the degree of market acceptance by physicians, patients, hospitals, cancer treatment centers, healthcare payors and others in the medical community necessary for commercial success.

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors and others in the medical community. For example, current cancer treatments like chemotherapy and radiation therapy are well established in the medical community, and physicians may continue to rely on these treatments. Most of our product candidates target mechanisms for which there are limited or no currently approved products, which may result in slower adoption by physicians, patients and payors. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and potential advantages compared to alternative treatments;
- our ability to offer our products for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the availability of coverage and adequate reimbursement from third party payors;
- the strength of marketing and distribution support; and
- the prevalence and severity of any side effects.

We may not be able to successfully commercialize our product candidates due to unfavorable pricing regulations or third-party coverage and reimbursement policies, which could make it difficult for us to sell our product candidates profitably.

Obtaining coverage and reimbursement approval for a product from a government or other third-party payor is a time-consuming and costly process, with uncertain results, that could require us to provide supporting scientific, clinical and cost effectiveness data for the use of our products to the payor. There may be significant delays in obtaining such coverage and reimbursement for newly approved products, and coverage may not be available, or may be more limited than the purposes for which the product is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that a product will be paid for in all cases or at a rate that covers our costs, including research, development, intellectual property, manufacture, sale and distribution expenses. Interim reimbursement levels for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the product and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost products and may be incorporated into existing payments for other services. Net prices for products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors, by any future laws limiting drug prices and by any future relaxation of laws that presently restrict imports of product from countries where they may be sold at lower prices than in the United States.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, there is no uniform policy among third-party payors for coverage and reimbursement. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting reimbursement policies, but also have their own methods and approval process apart from Medicare coverage and reimbursement determinations. Therefore, one third-party payor's determination to provide coverage for a product does not assure that other payors will also provide coverage for the product.

Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

We cannot be sure that reimbursement will be available for any product that we commercialize and, if coverage and reimbursement are available, what the level of reimbursement will be. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Reimbursement may impact the demand for, and the price of, any product for which we obtain marketing approval. Assuming we obtain coverage for a given product by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payors to reimburse all or part of the costs associated with those medications. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover all or a significant portion of the cost of our products. Therefore, coverage and adequate reimbursement is critical to a new product's acceptance. Coverage decisions may depend upon clinical and economic standards that disfavor new products when more established or lower cost therapeutic alternatives are already available or subsequently become available.

For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself may or may not be available. Instead, the hospital or administering physician may be reimbursed only for providing the treatment or procedure in which our product is used. Further, from time to time, the Centers for Medicare & Medicaid Services, or CMS revises the reimbursement systems used to reimburse health care providers, including the Medicare Physician Fee Schedule and Hospital Outpatient Prospective Payment System, which may result in reduced Medicare payments.

We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription medicines, medical devices and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the successful commercialization of new products. Further, the adoption and implementation of any future governmental cost containment or other health reform initiative may result in additional downward pressure on the price that we may receive for any approved product.

Additionally, we or collaborators may develop companion diagnostic tests for use with our product candidates. We, or our collaborators, will be required to obtain coverage and reimbursement for these tests separate and apart from the coverage and reimbursement we may seek for our product candidates. While we have not yet developed any companion diagnostic tests for our product candidates, if we do, there is significant uncertainty regarding our ability to obtain coverage and adequate reimbursement for the same reasons applicable to our product candidates.

Outside of the United States, many countries require approval of the sale price of a product before it can be marketed, and the pricing review period only begins after marketing or product licensing approval is granted. To obtain reimbursement or pricing approval in some of these countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product candidate in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and

negatively impact the revenue, if any, we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if such product candidates obtain marketing approval.

Our product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the Affordable Care Act, signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product.

We believe that any of our product candidates approved as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

If any approved products are subject to biosimilar competition sooner than we expect, we will face significant pricing pressure and our commercial opportunity will be limited.

If the market opportunities for any of our product candidates are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer.

We are focused initially on the development of treatments for cancer. Our projections of addressable patient populations that have the potential to benefit from treatment with our product candidates are based on estimates. If any of our estimates are inaccurate, the market opportunities for any of our product candidates could be significantly diminished and have an adverse material impact on our business.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we rely on third parties to research and develop and to manufacture our product candidates, we must share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's independent discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. For example, any academic institution that we may collaborate with will likely expect to

be granted rights to publish data arising out of such collaboration and any joint research and development programs may require us to share trade secrets under the terms of our research and development or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

If any of our product candidates are approved for marketing and commercialization and we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market our product candidates, we will be unable to successfully commercialize our product candidates if and when they are approved.

We have no sales, marketing or distribution capabilities or experience. To achieve commercial success for any approved product for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization, which would be expensive and time consuming, or outsource these functions to other third parties. In the future, we may choose to build a focused sales and marketing infrastructure to sell, or participate in sales activities with our collaborators for, some of our product candidates if and when they are approved.

There are risks involved with both establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize future products on our own include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product portfolios; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenue or the profitability of these product revenue to us are likely to be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. In entering into third-party marketing or distribution arrangements, any revenue we receive will depend upon the efforts of the third parties and we cannot assure you that such third parties will establish adequate sales and distribution capabilities or devote the necessary resources and attention to sell and market any future products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

Even if we obtain FDA approval of any of our product candidates, we may never obtain approval or commercialize such products outside of the United States, which would limit our ability to realize their full market potential.

In order to market any products outside of the United States, we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval procedures vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approvals could result in significant delays, difficulties and costs for us and may require

additional preclinical studies or clinical trials which would be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. Satisfying these and other regulatory requirements is costly, time consuming, uncertain and subject to unanticipated delays. In addition, our failure to obtain regulatory approval in any country may delay or have negative effects on the process for regulatory approval in other countries. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, our ability to realize the full market potential of our products will be harmed.

Risks Related to Our In-Licenses and Other Strategic Agreements

We are currently party to several in-license agreements under which we acquired rights to use, develop, manufacture and/or commercialize certain of our platform technologies and resulting product candidates. If we breach our obligations under these agreements, we may be required to pay damages, lose our rights to these technologies or both, which would adversely affect our business and prospects.

We rely, in part, on license and other strategic agreements, which subject us to various obligations, including diligence obligations with respect to development and commercialization activities, payment obligations for achievement of certain milestones and royalties on product sales, negative covenants and other material obligations. For example, with respect to P-BCMA-101 and P-PSMA-101, we have licensed Centyrin binders under an agreement with Janssen Biotech Inc., or Janssen, with respect to P-BCMA-ALLO1, we have licensed heavy-chain-only binders under an agreement with TeneoBio, Inc., or TeneoBio, with respect to P-MUC1C-ALLO1, we have licensed potential binders under our agreements with Genus Oncology, LLC, or Genus, and TeneoBio, with respect to P-PSMA-ALLO1 and our dual CAR programs we have licensed and may continue to license binders under our agreements with TeneoBio, and with respect to our Cas-CLOVER gene editing technology, which we use in the manufacture of P-BCMA-ALLO1, P-MUC1C-ALLO1, P-PSMA-ALLO1 and future allogeneic products, we have licensed certain intellectual property under an agreement with Helmholtz-Zentrum München—Deutsches Forschungszentrum für Gesundheit und Umwelt GmbH. If we fail to comply with the obligations under our license agreements, including as a result of COVID-19 impacting our operations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages and our licensors may have the right to terminate the license. If our license agreements are terminated, we may not be able to develop, manufacture, market or sell the products covered by our agreements and those being tested or approved in combination with such products. Such an occurrence could materially adversely affect the value of the product candidates being developed under any such agreement.

In addition, the agreements under which we license intellectual property or technology to or from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

Our business also would suffer if any current or future licensors fail to abide by the terms of the license, if the licensors fail to enforce licensed patents against infringing third parties, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights.

In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in products that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize products, we may be unable to achieve or maintain profitability.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant research programs or product candidates and our business, financial condition, results of operations and prospects could suffer.

We may not realize the benefits of any acquisitions, in-license or strategic alliances that we enter into.

We have entered into in-license agreements with multiple licensors and in the future may seek and form strategic alliances, create joint ventures or collaborations, or enter into acquisitions or additional licensing arrangements with third parties that we believe will complement or augment our existing technologies and product candidates.

These transactions can entail numerous operational and financial risks, including exposure to unknown liabilities, disruption of our business and diversion of our management's time and attention in order to manage a collaboration or develop acquired products, product candidates or technologies, incurrence of substantial debt or dilutive issuances of equity securities to pay transaction consideration or costs, higher than expected collaboration, acquisition or integration costs, write-downs of assets or goodwill or impairment charges, increased amortization expenses, difficulty and cost in facilitating the collaboration or combining the operations and personnel of any acquired business, impairment of relationships with key suppliers, manufacturers or customers of any acquired business due to changes in management and ownership and the inability to retain key employees of any acquired business. As a result, if we enter into acquisition or in-license agreements or strategic partnerships, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture, or if there are materially adverse impacts on our or the counterparty's operations resulting from COVID-19, which could delay our timelines or otherwise adversely affect our business. We also cannot be certain that, following a strategic transaction or license, we will achieve the revenue or specific net income that justifies such transaction or such other benefits that led us to enter into the arrangement.

We may wish to form collaborations in the future with respect to our product candidates, but may not be able to do so or to realize the potential benefits of such transactions, which may cause us to alter or delay our development and commercialization plans.

The development and potential commercialization of our product candidates will require substantial additional capital to fund expenses. We may, in the future, decide to collaborate with other biopharmaceutical companies for the development and potential commercialization of those product candidates, including in territories outside the United States or for certain indications. We will face significant competition in seeking appropriate collaborators. We may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our product candidates because they may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy. If and when we collaborate with a third-party for development and commercialization of a product candidate, we can expect to relinquish some or all of the control over the future success of that product candidate to the third-party. Our ability to reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of our technologies, product candidates and market opportunities. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. We may also be restricted under any license agreements from entering into agreements on certain terms or at all with potential collaborators.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators and changes to the strategies of the combined company. As a result, we may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of such product candidate, reduce or delay one or more of our other development programs, delay the potential commercialization or reduce the scope of any planned sales or marketing activities for such product candidate, or increase our expenditures and undertake development, manufacturing or commercialization activities at our own expense. If we elect to increase our expenditures to fund development,

manufacturing or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

Our product candidates may also require specific components to work effectively and efficiently, and rights to those components may be held by others. We may be unable to in-license any compositions, methods of use, processes or other third party intellectual property rights from third parties that we identify. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, which would harm our business. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

Risks Related to Our Industry and Business Operations

The COVID-19 pandemic continues to adversely impact our business, including our clinical trials, supply chain and business development activities.

In March 2020, the World Health Organization made the assessment that a novel strain of coronavirus, SARS-CoV-2, a novel strain of coronavirus, commonly referred to as COVID-19 had become a global pandemic. In March 2020, the United States declared the COVID-19 pandemic a national emergency and many states and municipalities in the United States have taken aggressive actions to reduce the spread and ameliorate the impact of the disease, including limiting non-essential gatherings of people and non-essential travel, ordering certain businesses and government agencies to cease non-essential operations at physical locations and issuing “shelter-in-place” orders which direct individuals to shelter at their places of residence (subject to limited exceptions) and have also implemented multi-step policies with the goal of re-opening such states and municipalities. As a result of these actions and in an effort to ensure the safety of employees during the pandemic, a majority of our employees are at least partially currently telecommuting, which has impacted certain of our operations and may continue to do so over the long term. We may experience further limitations on employee resources in the future, including because of sickness of employees or their families. The effects of government actions and our own policies and those of third parties to reduce the spread of COVID-19 continues to negatively impact productivity and slow down or delay our ongoing and future clinical trials, preclinical studies and research and development activities, and may cause disruptions to our supply chain and impair our ability to execute our business development strategy. In the event that government authorities were to enhance current restrictions, our employees who currently are not telecommuting may no longer be able to access our facilities, and our operations may be further limited or curtailed.

As COVID-19 continues to spread, we expect to experience ongoing disruptions that could severely impact our business, preclinical studies and clinical trials, including:

- delays in receiving approval from local regulatory authorities to initiate our planned clinical trials;
- delays or difficulties in enrolling and maintaining patients in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- delays in clinical sites receiving the supplies and materials needed to conduct our clinical trials, including interruption in global shipping that may affect the transport of clinical trial materials;
- changes in local regulations as part of a response to the COVID-19 outbreak which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue the clinical trials altogether;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others, or interruption of clinical trial subject visits and study procedures, the occurrence of which could affect the integrity of clinical trial data;

- interruption or delays in the operations of the FDA or other regulatory authorities, which may impact review and approval timelines;
- risk that participants enrolled in our clinical trials will acquire COVID-19 while the clinical trial is ongoing, which could impact the results of the clinical trial, including by increasing the number of observed adverse events; and
- refusal of the FDA to accept data from clinical trials in affected geographies.

These and other disruptions in our operations and the global economy could negatively impact our business, operating results and financial condition.

Our clinical trials have been, and we expect will continue to be, affected by the COVID-19 pandemic. For example, some of our clinical trial sites have slowed down or stopped further enrollment of new patients in clinical trials, denied access to site monitors and otherwise curtailed certain operations. Similarly, our ability to recruit and retain principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19, may be adversely impacted. Our ongoing or planned clinical trials may also be impacted by interruptions or delays in the operations of the FDA and comparable foreign regulatory agencies. We and our CROs have also made certain adjustments to the operation of our trials in an effort to ensure the monitoring and safety of patients and minimize risks to trial integrity during the pandemic in accordance with the guidance issued by the FDA, and may need to make further adjustments in the future. Many of these adjustments are new and untested, may not be effective, and may have unforeseen effects on the enrollment, progress and completion of these trials and the findings from these trials. These events could delay our clinical trials, increase the cost of completing our clinical trials and negatively impact the integrity, reliability or robustness of the data from our clinical trials.

In addition, quarantines, shelter-in-place and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could impact personnel at third-party manufacturing facilities upon which we rely, or the availability or cost of materials, which could disrupt the supply chain for our product candidates. To the extent our suppliers and service providers are unable to comply with their obligations under our agreements with them or they are otherwise unable to deliver or are delayed in delivering goods and services to us due to the COVID-19 pandemic, our ability to continue meeting clinical supply demand for our product candidates or otherwise advancing development of our product candidates may become impaired.

Furthermore, quarantines, shelter-in-place and similar government orders related to COVID-19 or other infectious diseases could cause validation and qualification of our pilot manufacturing facility to be delayed significantly. In addition, even though the pilot manufacturing facility is operational, such government orders could prevent us from operating the facility as intended. These events could delay our ability to manufacture clinical-scale materials for certain of our product candidates and otherwise delay the development of certain of our product candidates.

The spread of COVID-19 and actions taken to reduce its spread may also materially affect us economically. While the potential economic impact brought by, and the duration of, the COVID-19 pandemic may be difficult to assess or predict, there could be a significant disruption of global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity and financial position. In addition, the trading prices for other biopharmaceutical companies have been highly volatile as a result of the COVID-19 pandemic. As a result, we may face difficulties raising capital through sales of our common stock or such sales may be on unfavorable terms.

COVID-19 and actions taken to reduce its spread continue to rapidly evolve. The extent to which COVID-19 may impede the development of our product candidates, reduce the productivity of our employees, disrupt our supply chains, delay our clinical trials, reduce our access to capital or limit our business development activities, will depend on future developments, which are highly uncertain and cannot be predicted with confidence. To the extent the COVID-19 pandemic adversely affects our business and financial results, it may also have the effect of heightening many of the other risks described in this “Risk Factors” section, such as those relating to the timing and results of our clinical trials and our financing needs.

We face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use of our product candidates harms patients or is perceived to harm patients even when such harm is unrelated to our product candidates, our regulatory approvals could be revoked or otherwise negatively impacted and we could be subject to costly and damaging product liability claims.

The use of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- impairment of our business reputation;
- withdrawal of clinical trial participants;
- costs due to related litigation;
- distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- the inability to commercialize our product candidates; and
- decreased demand for our product candidates, if approved for commercial sale.

We carry product liability insurance of \$5.0 million per occurrence and \$5.0 million aggregate limit. We believe our product liability insurance coverage is sufficient in light of our current clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If and when we obtain marketing approval for product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs or medical treatments that had unanticipated adverse effects. A successful product liability claims, or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

Patients with cancer and other diseases targeted by our product candidates are often already in severe and advanced stages of disease and have both known and unknown significant pre-existing and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may be related to our product candidates, such as the patient death that occurred in our Phase 1 P-PSMA-101 trial. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to our products, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may interrupt our sales efforts, delay our regulatory approval process in other countries, or impact and limit the type of regulatory approvals our product candidates receive or maintain. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, scientific and medical personnel. The loss of the services of any of our executive officers, other key employees, and other scientific and medical advisors, and our inability to find suitable replacements could result in delays in product development and harm our business.

We conduct substantially all of our operations at our facilities in San Diego. This region is headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel in our market is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock options that vest over time. The value to employees of stock options that vest over time may be significantly affected by movements in our stock price that are beyond our control and may at any time be insufficient to counteract more lucrative offers from other companies. Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have employment agreements with certain of our key employees, these employment agreements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. Other than for Dr. Ostertag, our Chief Executive Officer, we do not maintain “key person” insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level and senior managers as well as junior, mid-level and senior scientific and medical personnel.

We expect to expand our development, regulatory and operational capabilities and, as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of December 31, 2020, we had 206 employees. As we advance our research and development programs, we may be required to further increase the number of our employees and the scope of our operations, particularly in the areas of clinical development, manufacturing, quality, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage any future growth, we must:

- identify, recruit integrate, maintain and motivate additional qualified personnel;
- manage our development efforts effectively, including the initiation and conduct of clinical trials for our product candidates, both as monotherapy and in combination with other intra-portfolio product candidates; and
- improve our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to develop, manufacture and commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert financial and other resources, and a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time, to managing these growth activities.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

We face substantial competition, which may result in others discovering, developing or commercializing products more quickly or marketing them more successfully than us.

The development and commercialization of new products is highly competitive. We compete in the segments of the pharmaceutical, biotechnology and other related markets that develop immunotherapies for the treatment of cancer. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop or that would render any products that we may develop obsolete or non-competitive. Our competitors also may obtain marketing approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Moreover, with the proliferation of new drugs and therapies into oncology, we expect to face increasingly intense competition as new technologies become available. If we fail to stay at the forefront of technological change, we may be unable to compete effectively. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the

future. The highly competitive nature of and rapid technological changes in the biotechnology and pharmaceutical industries could render our product candidates or our technology obsolete, less competitive or uneconomical.

Other products in the same class as some of our product candidates have already been approved or are further along in development. As more product candidates within a particular class of biopharmaceutical products proceed through clinical development to regulatory review and approval, the amount and type of clinical data that may be required by regulatory authorities may increase or change. Consequently, the results of our clinical trials for product candidates in those class will likely need to show a risk benefit profile that is competitive with or more favorable than those products and product candidates in order to obtain marketing approval or, if approved, a product label that is favorable for commercialization. If the risk benefit profile is not competitive with those products or product candidates, we may have developed a product that is not commercially viable, that we are not able to sell profitably or that is unable to achieve favorable pricing or reimbursement. In such circumstances, our future product revenue and financial condition would be materially and adversely affected.

Specifically, there are many companies pursuing a variety of approaches to CAR-T therapies, including Adaptimmune Therapeutics plc, Allogene, Inc., Astellas Pharma, Inc., Autolus Ltd., Bellicum Pharmaceuticals Inc., Bluebird Bio, Inc., Cellectis S.A., Janssen Pharmaceuticals Inc., Juno Therapeutics, Inc. (acquired by Celgene Corporation, now a Bristol-Meyers Squibb company), Kite Pharma, Inc. (a Gilead Sciences, Inc. company), Legend Biotech Corporation, Novartis AG and Takeda Inc. Immunotherapy and gene therapy approaches are further being pursued by many smaller biotechnology companies as well as larger pharmaceutical companies. We also face competition from non-cell-based or other gene therapy treatments offered by companies such as Amgen Inc., AstraZeneca plc, Beam Therapeutics, Inc, Bristol-Myers Squibb Company, F. Hoffman-La Roche AG, Generation Bio, Inc., GlaxoSmithKline plc, Merck & Co., Inc. PassageBio, Inc. and Pfizer Inc. Many of our competitors, either alone or with their collaboration partners, have substantially greater financial, technical and other resources, such as larger research and development staff and/or greater expertise in research and development, manufacturing, preclinical testing and conducting clinical trials.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and subject enrollment for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

The key competitive factors affecting the success of all of our programs are likely to be their efficacy, safety, convenience, and availability of reimbursement. If we are not successful in developing, commercializing and achieving higher levels of reimbursement than our competitors, we will not be able to compete against them and our business would be materially harmed.

We or the third parties upon whom we depend may be adversely affected by earthquakes, fires or other natural disasters.

Our headquarters, main research facility and pilot manufacturing facility are located in San Diego, California, which in the past has experienced severe earthquakes and fires. If these earthquakes, fires, other natural disasters, terrorism and similar unforeseen events beyond our control prevented us from using all or a significant portion of our headquarters or research facility, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. We do not have a disaster recovery or business continuity plan in place and may incur substantial expenses as a result of the absence or limited nature of our internal or third-party service providers' disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business. Furthermore, integral parties in our supply chain are operating from single sites, increasing their vulnerability to natural disasters or other sudden, unforeseen and severe adverse events. If such an event were to affect our supply chain, it could have a material adverse effect on our ability to conduct our clinical trials, our development plans, business, financial condition or results of operations.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future, and we may never achieve profitability. Unused U.S. federal net operating losses, or NOLs, for taxable years beginning before January 1, 2018, may be carried forward to offset future taxable income, if any, until such unused NOLs expire. Under legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, or the Tax Act, as modified by legislation enacted on March 27, 2020, entitled the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, U.S. federal NOLs incurred in taxable years beginning after December 31, 2017, can be carried forward indefinitely, but the deductibility of such U.S. federal NOLs in taxable years beginning after December 31, 2020, is limited to 80% of taxable income. It is uncertain if and to what extent various states will conform to the Tax Act or the CARES Act.

As of December 31, 2020, we had \$23.3 million of U.S. federal NOLs that will begin to expire in 2032, and \$205.2 million of U.S. federal NOLs that can be carried forward indefinitely under current law. As of December 31, 2020, we also had aggregate U.S. federal orphan drug credits and research and development, or R&D, credits of approximately \$17.7 million. Our NOL carryforwards and R&D credits are subject to review and possible adjustment by the U.S. and state tax authorities.

In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, and corresponding provisions of state law, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50 percentage point change (by value) in its equity ownership over a three-year period, the corporation’s ability to use its pre-change NOL carryforwards, R&D credits and certain other tax attributes to offset its post-change income or taxes may be limited. This could limit the amount of NOLs, R&D credit carryforwards or other applicable tax attributes that we can utilize annually to offset future taxable income or tax liabilities. Subsequent ownership changes and changes to the U.S. tax rules in respect of the utilization of NOLs, R&D credits and other applicable tax attributes carried forward may further affect the limitation in future years. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. For example, California imposed limits on the usability of California state net operating losses to offset taxable income in tax years beginning after 2019 and before 2023. As a result, if we earn net taxable income, we may be unable to use all or a material portion of our net operating loss carryforwards and other tax attributes, which could potentially result in increased future tax liability to us and adversely affect our future cash flows.

Changes in healthcare law and implementing regulations, as well as changes in healthcare policy, may impact our business in ways that we cannot currently predict, and may have a significant adverse effect on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any product candidates for which we obtain marketing approval. Among policy makers and payors in the United States and elsewhere, including in the EU, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives.

The Affordable Care Act, substantially changed the way healthcare is financed by both the government and private insurers, and significantly impacts the U.S. pharmaceutical industry. The Affordable Care Act, among other things: (1) introduced a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for certain drugs and biologics that are inhaled, infused, instilled, implanted or injected and not generally dispensed through retail community pharmacies; (2) increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program; (3) established a branded prescription drug fee that pharmaceutical manufacturers of branded prescription drugs must pay to the federal government; (4) expanded the list of covered entities eligible to participate in the 340B drug pricing program by adding new entities to the program; (5) established a new Medicare Part D coverage gap discount program, in which manufacturers must now agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer’s outpatient drugs to

be covered under Medicare Part D; (6) extended manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations; (7) expanded eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for individuals with income at or below 133% of the federal poverty level, thereby potentially increasing manufacturers' Medicaid rebate liability; (8) created a licensure framework for follow on biologic products; (9) established a Center for Medicare Innovation at CMS, to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending; and (10) created a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research.

There remain judicial and Congressional challenges to certain aspects of the Affordable Care Act, as well as efforts by the Trump administration to repeal or replace and replace certain aspects of the Affordable Care Act. While Congress has not passed comprehensive repeal legislation, the Tax Act includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the Affordable Care Act's mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminated the health insurer tax. Further, the Bipartisan Budget Act of 2018, or the BBA, among other things, amends the Affordable Care Act, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole". On December 14, 2018, a Texas U.S. District Court Judge ruled that the Affordable Care Act is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the Affordable Care Act are invalid as well. The U.S. Supreme Court is currently reviewing this case, but it is unknown when a decision will be reached. Although the U.S. Supreme Court has yet to rule on the constitutionality of the ACA, on January 28, 2021, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021 through May 15, 2021 for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructs certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. It is unclear how the Supreme Court ruling, other such litigation, and the healthcare reform measures of the Biden administration will impact the ACA and our business or financial condition.

Other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. These changes include aggregate reductions to Medicare payments to providers of 2% per fiscal year pursuant to the Budget Control Act of 2011, which began in 2013 and, due to legislative amendments to the statute, including the BBA and the CARES Act, will remain in effect through 2030 unless additional Congressional action is taken. These reductions have been suspended from May 1, 2020 through March 31, 2021 due to the COVID-19 pandemic. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Additional changes that may affect our business include the expansion of new programs such as Medicare payment for performance initiatives for physicians, also referred to as the Quality Payment Program, under the Medicare Access and CHIP Reauthorization Act of 2015. In November 2019, CMS issued a final rule finalizing the changes to the Quality Payment Program. At this time, it is unclear how the introduction of the Quality Payment Program will impact overall physician reimbursement under the Medicare Program. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. In addition, new laws may result in additional reductions in Medicare and other healthcare funding, which may materially adversely affect customer demand and affordability for our products and, accordingly, the results of our financial operations.

Also, there has been heightened governmental scrutiny recently over the manner in which drug manufacturers set prices for their marketed products, which have resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program

reimbursement methodologies for drug products. At the federal level, the Trump administration used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives. For example, on July 24, 2020 and September 13, 2020, President Trump announced several executive orders related to prescription drug pricing that attempt to implement several of the Trump administration's proposals. As a result, the FDA released a final rule, effective November 30, 2020, implementing a portion of the importation executive order providing guidance for states to build and submit importation plans for drugs from Canada. Further, on November 20, 2020, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The implementation of the rule has been delayed by the Biden administration from January 1, 2022 to January 1, 2023 in response to ongoing litigation. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a new safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers, the implementation of which have also been delayed pending review by the Biden administration until March 22, 2021. On November 20, 2020, CMS issued an interim final rule implementing President Trump's Most Favored Nation executive order, which would tie Medicare Part B payments for certain physician-administered drugs to the lowest price paid in other economically advanced countries, effective January 1, 2021. On December 28, 2020, the United States District Court in Northern California issued a nationwide preliminary injunction against implementation of the interim final rule. However, it is unclear whether the Biden administration will work to reverse these measures or pursue similar policy initiatives.

We expect that these and other healthcare reform measures that may be adopted in the future may result in more rigorous coverage criteria and lower reimbursement and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government-funded programs may result in a similar reduction in payments from private payors. The implementation of cost-containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drugs once marketing approval is obtained.

Further, it is possible that additional governmental action will be taken in response to the COVID-19 pandemic.

In the European Union, coverage and reimbursement status of any product candidates for which we obtain regulatory approval are provided for by the national laws of EU Member States. The requirements may differ across the EU Member States. Also, at national level, actions have been taken to enact transparency laws regarding payments between pharmaceutical companies and health care professionals.

We are subject to applicable fraud and abuse, transparency, government price reporting, and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Healthcare providers and third-party payors will play a primary role in the recommendation and prescription of any future product candidates we may develop and any product candidates for which we obtain marketing approval. Our current and future arrangements with clinical investigators, third-party payors, healthcare provider and customers expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may affect the business or financial arrangements and relationships through which we research, market, sell and distribute our products. The laws that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits any person or entity from, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of an item or service reimbursable, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. The term "remuneration" has been broadly interpreted to include anything of value. The federal Anti-Kickback Statute has also been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, and purchasers, on the other the other hand. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, but these exceptions and safe harbors are narrowly drawn. Practices that are alleged to be intended to induce prescribing,

purchases or recommendations, or include any payments of more than fair market value, may be subject to scrutiny if they do not qualify for an exception or safe harbor;

- federal civil and criminal false claims laws, such as the civil False Claims Act, or FCA, which can be enforced by private citizens through civil qui tam actions, and civil monetary penalty laws prohibits individuals or entities from, among other things, knowingly presenting, or causing to be presented, false, fictitious or fraudulent claims for payment of federal funds, and knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to avoid, decrease or conceal an obligation to pay money to the federal government. For example, pharmaceutical companies have been prosecuted under the FCA in connection with, among other things their alleged off-label promotion of drugs, engaging in improper consulting arrangements with physicians, concealing price concessions in the pricing information submitted to the government for government price reporting purposes, and providing free product to customers with the expectation that the customers would bill federal health care programs for the product. In addition, a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA. As a result of a modification made by the Fraud Enforcement and Recovery Act of 2009, a claim includes “any request or demand” for money or property presented to the U.S. government. In addition, manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to “cause” the submission of false or fraudulent claims;
- HIPAA which, among other things, imposes criminal liability for executing or attempting to execute a scheme to defraud any healthcare benefit program, including private third-party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and creates federal criminal laws that prohibit knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement or representation, or making or using any false writing or document knowing the same to contain any materially false, fictitious or fraudulent statement or entry in connection with the delivery of or payment for healthcare benefits, items or services;
- HIPAA, as amended by HITECH and their implementing regulations, which imposes privacy, security and breach reporting obligations with respect to individually identifiable health information upon covered entities, including certain healthcare providers, health plans, and healthcare clearinghouses, as well as their respective business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, and their subcontractors that use, disclose or otherwise process individually identifiable health information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in U.S. federal courts to enforce the federal HIPAA laws and seek attorneys’ fees and costs associated with pursuing federal civil actions;
- the federal transparency requirements under the Physician Payments Sunshine Act, created under the Affordable Care Act, which requires, among other things, certain manufacturers of drugs, devices, biologics and medical supplies reimbursed under Medicare, Medicaid, or the Children’s Health Insurance Program to report to CMS information related to payments and other transfers of value provided to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals and certain physician ownership and investment interests, including such ownership and investment interests held by a physician’s immediate family members. Beginning in 2022, applicable manufacturers also will be required to report such information regarding its relationships with physician assistants, nurse practitioners, clinical nurse specialists, anesthesiologist assistants, certified registered nurse anesthetists and certified nurse midwives during the previous year;
- analogous state, local and foreign laws and regulations, such as anti-kickback and false claims laws, that may impose similar or more prohibitive restrictions, and may apply to items or services reimbursed by any non-governmental third-party payors, including private insurers; and
- state and foreign laws that require pharmaceutical companies to implement compliance programs, comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or to track and report gifts, compensation and other remuneration provided to physicians and other health care providers, state and local laws that

require the registration of pharmaceutical sales representatives, and other federal, state and foreign laws that govern the privacy and security of health information or personally identifiable information in certain circumstances, including state health information privacy and data breach notification laws which govern the collection, use, disclosure, and protection of health-related and other personal information, many of which differ from each other in significant ways and often are not pre-empted by HIPAA, thus requiring additional compliance efforts.

We may also be subject to federal and state consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers. We have entered into consulting and scientific advisory board arrangements with physicians and other healthcare providers, some of which include provisions of stock options, including some who could influence the use of our product candidates, if approved. Because of the complex and far-reaching nature of these laws, regulatory agencies may view these transactions as prohibited arrangements that must be restructured, or discontinued, or for which we could be subject to other significant penalties. We could be adversely affected if regulatory agencies interpret our financial relationships with providers who may influence the ordering of and use our product candidates, if approved, to be in violation of applicable laws.

Federal and state enforcement bodies have continued their scrutiny of interactions between healthcare companies and healthcare providers, which has led to significant investigations, prosecutions, convictions and settlements in the healthcare industry. Responding to investigations can be time- and resource-consuming and can divert management's attention from the business. Any such investigation or settlement could increase our costs or otherwise have an adverse effect on our business.

Ensuring that our business arrangements with third parties comply with applicable healthcare laws and regulations will likely be costly. If our operations are found to be in violation of any of these laws or any other current or future governmental laws and regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, contractual damages, reputational harm, diminished profits and future earnings, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could substantially disrupt our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain sufficient intellectual property protection for our platform technologies and product candidates, or if the scope of the intellectual property protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our products may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our platform technologies and product candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel discoveries and technologies that are important to our business. Our pending and future patent applications may not result in patents being issued which protect our product candidates or their intended uses or which effectively prevent others from commercializing competitive technologies, products or product candidates.

Obtaining and enforcing patents is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications or maintain and/or enforce patents that may issue based on our patent applications, at a reasonable cost or in a timely manner, including as a result of the COVID-19 pandemic impacting our or our licensors' operations. It is also possible that we will fail to identify patentable aspects of our research and development results before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, contract

research organizations, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach these agreements and disclose such results before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

Composition of matter patents for biological and pharmaceutical products such as CAR-based product candidates often provide a strong form of intellectual property protection for those types of products, as such patents provide protection without regard to any method of use. We cannot be certain that the claims in our pending patent applications covering composition of matter of our product candidates will be considered patentable by the United States Patent and Trademark Office, or USPTO, or by patent offices in foreign countries, or that the claims in any of our issued patents will be considered valid and enforceable by courts in the United States or foreign countries. Method of use patents protect the use of a product for the specified method. This type of patent does not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their product for our targeted indications, physicians may prescribe these products “off-label.” Although off-label prescriptions may infringe or contribute to the infringement of method of use patents, the practice is common and such infringement is difficult to prevent or prosecute.

The patent position of biopharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation, resulting in court decisions, including Supreme Court decisions, which have increased uncertainties as to the ability to enforce patent rights in the future. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States, or vice versa.

Further, we may not be aware of all third-party intellectual property rights potentially relating to our product candidates or their intended uses, and as a result the impact of such third-party intellectual property rights upon the patentability of our own patents and patent applications, as well as the impact of such third-party intellectual property upon our freedom to operate, is highly uncertain. Patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in post-grant review procedures, oppositions, derivations, reexaminations, or *inter partes* review proceedings, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated, or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. Any failure to obtain or maintain patent protection with respect to our product candidates could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop and market our products.

We cannot guarantee that any of our patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction.

The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent’s prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect, which may negatively impact our ability to market our products. We may incorrectly determine that our products are not covered by a third-party patent or may incorrectly predict whether a third-party’s pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the

United States or abroad that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our product candidates. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our products.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty and other obligations on us. If we fail to comply with our obligations under these agreements, including due to the impact of the COVID-19 pandemic on our business operations, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend considerable time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current product candidates or future products, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

In many cases, patent prosecution of our licensed technology is controlled solely by the licensor. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, including due to the impact of the COVID-19 pandemic on our licensors' business operations, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business and our competitors could market competing products using the intellectual property. In certain cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues and is complicated by the rapid pace of scientific discovery in our industry. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. We are generally also subject to all of the same risks with respect to protection of intellectual property that we license as we are for intellectual property that we own, which are described herein. If we or our licensor fail to adequately protect this intellectual property, our ability to commercialize products could suffer.

In the future, we may need to obtain additional licenses of third-party technology that may not be available to us or are available only on commercially unreasonable terms, and which may cause us to operate our business in a more costly or otherwise adverse manner that was not anticipated.

We currently have rights to intellectual property covering our product candidates and other proprietary technologies. Other pharmaceutical companies and academic institutions may also have filed or are planning to file patent applications potentially relevant to our business. From time to time, in order to avoid infringing these third-party patents, we may be required to license technology from additional third parties to further develop or commercialize our product candidates. Should we be required to obtain licenses to any third-party technology, including any such patents required to manufacture, use or sell our product candidates, such licenses may not be available to us on commercially reasonable terms, or at all. The inability to obtain any third-party license required to develop or commercialize any of our product candidates could cause us to abandon any related efforts, which could seriously harm our business and operations.

The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us.

Moreover, some of our owned and in-licensed patents or patent applications or future patents are or may be co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us. Furthermore, our owned and in-licensed patents may be subject to a reservation of rights by one or more third parties. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

We cannot ensure that patent rights relating to inventions described and claimed in our pending patent applications will issue or that patents based on our patent applications will not be challenged and rendered invalid and/or unenforceable.

We have pending U.S. and foreign patent applications in our portfolio; however, we cannot predict:

- if and when patents may issue based on our patent applications;
- the scope of protection of any patent issuing based on our patent applications;
- whether the claims of any patent issuing based on our patent applications will provide protection against competitors;
- whether or not third parties will find ways to invalidate or circumvent our patent rights;
- whether or not others will obtain patents claiming aspects similar to those covered by our patents and patent applications;
- whether we will need to initiate litigation or administrative proceedings to enforce and/or defend our patent rights which will be costly whether we win or lose; and/or
- whether the patent applications that we own, or in-license will result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries.

We cannot be certain that the claims in our pending patent applications directed to our product candidates and/or technologies will be considered patentable by the USPTO or by patent offices in foreign countries. There can be no assurance that any such patent applications will issue as granted patents. One aspect of the determination of patentability of our inventions depends on the scope and content of the "prior art," information that was or is deemed available to a person of skill in the relevant art prior to the priority date of the claimed invention. There may be prior art of which we are not aware that may affect the patentability of our patent claims or, if issued, affect the

validity or enforceability of a patent claim. Even if the patents do issue based on our patent applications, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, patents in our portfolio may not adequately exclude third parties from practicing relevant technology or prevent others from designing around our claims. If the breadth or strength of our intellectual property position with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop and threaten our ability to commercialize our product candidates. In the event of litigation or administrative proceedings, we cannot be certain that the claims in any of our issued patents will be considered valid by courts in the United States or foreign countries.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make product candidates that are similar to ours but that are not covered by the claims of the patents that we own or have exclusively licensed;
- we or our licensors or future collaborators might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or have exclusively licensed;
- we or our licensors or future collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or have exclusively licensed may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- we cannot predict the scope of protection of any patent issuing based on our patent applications, including whether the patent applications that we own or in-license will result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries;
- the claims of any patent issuing based on our patent applications may not provide protection against competitors or any competitive advantages, or may be challenged by third parties;
- if enforced, a court may not hold that our patents are valid, enforceable and infringed;
- we may need to initiate litigation or administrative proceedings to enforce and/or defend our patent rights which will be costly whether we win or lose;
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property;
- we may fail to adequately protect and police our trademarks and trade secrets; and
- the patents of others may have an adverse effect on our business, including if others obtain patents claiming subject matter similar to or improving that covered by our patents and patent applications.

Should any of these events occur, they could significantly harm our business, results of operations and prospects.

If we are sued for infringing intellectual property rights of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing our product candidates.

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our product candidates without infringing the intellectual property and other proprietary rights of third parties. Third parties may allege that we have infringed or misappropriated their intellectual property. For example, in early 2019, we received a letter from a third party alleging that we have used materials received from the third party in an unauthorized manner and stating a belief that we will infringe certain patents relating to the use of a safety switch in our CAR-T products. While we have denied that we used any of the third party's materials in an unauthorized manner and believe that the patents will not be infringed, are invalid, or both, we cannot predict whether the third party will persist in its allegations or whether litigation will ensue. Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time consuming and, even if resolved in our favor, is likely to divert significant resources from our core business, including distracting our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the market price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to our product candidates. We cannot assure you that our product candidates and other proprietary technologies we may develop will not infringe existing or future patents owned by third parties. Third parties may assert infringement claims against us based on existing or future intellectual property rights. We may not be aware of patents that have already been issued and that a third party, for example, a competitor in the fields in which we are developing our product candidates, might assert are infringed by our current or future product candidates, including claims to compositions, formulations, methods of manufacture or methods of use or treatment that cover our product candidates. It is also possible that patents owned by third parties of which we are aware, but which we do not believe are relevant to our product candidates and other proprietary technologies we may develop, could be found to be infringed by our product candidate. In addition, because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. Our competitors in both the United States and abroad, many of which have substantially greater resources and have made substantial investments in patent portfolios and competing technologies, may have applied for or obtained or may in the future apply for and obtain, patents that will prevent, limit or otherwise interfere with our ability to make, use and sell our product candidates. The pharmaceutical and biotechnology industries have produced a considerable number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we were sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity may be difficult. For example, in the United States, proving invalidity in court requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents, and there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on our business and operations. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third-party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third-party in order to use the infringing technology

and continue developing, manufacturing or marketing the infringing product candidate. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, and could divert the time and attention of our technical personnel and management, cause development delays, and/or require us to develop non-infringing technology, which may not be possible on a cost-effective basis, any of which could materially harm our business. In the event of a successful claim of infringement against us, we may have to pay substantial monetary damages, including treble damages and attorneys' fees for willful infringement, pay royalties and other fees, redesign our infringing drug or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors or other third parties may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Our pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or insufficient written description. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention, or decide that the other party's use of our patented technology falls under the safe harbor to patent infringement under 35 U.S.C. §271(e)(1). An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock. Moreover, we cannot assure you that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

Because of the expense and uncertainty of litigation, we may conclude that even if a third-party is infringing our issued patent, any patents that may be issued as a result of our pending or future patent applications or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our stockholders, or it may be otherwise impractical or undesirable to enforce our intellectual property against some third parties. Our competitors or other third parties may be able to sustain the costs of complex patent litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution. In addition, the uncertainties associated with litigation could compromise our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, in-license needed technology or other product candidates, or enter into development partnerships that would help us bring our product candidates to market.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.

We could in the future be subject to claims that we or our employees have inadvertently or otherwise used or disclosed alleged trade secrets or other confidential information of former employers or competitors. Although we try to ensure that our employees and consultants do not use the intellectual property, proprietary information, know-how or trade secrets of others in their work for us, we may become subject to claims that we caused an employee to breach the terms of his or her non-competition or non-solicitation agreement, or that we or these individuals have, inadvertently or otherwise, used or disclosed the alleged trade secrets or other proprietary information of a former employer or competitor.

While we may litigate to defend ourselves against these claims, even if we are successful, litigation could result in substantial costs and could be a distraction to management. If our defenses to these claims fail, in addition to requiring us to pay monetary damages, a court could prohibit us from using technologies or features that are essential to our product candidates, if such technologies or features are found to incorporate or be derived from the trade secrets or other proprietary information of the former employers. Moreover, any such litigation or the threat thereof may adversely affect our reputation, our ability to form strategic alliances or sublicense our rights to collaborators, engage with scientific advisors or hire employees or consultants, each of which would have an adverse effect on our business, results of operations and financial condition. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

We may not be able to protect our intellectual property rights throughout the world.

Patents are of national or regional effect, and filing, prosecuting and defending patents on all of our product candidates throughout the world would be prohibitively expensive. As such, our intellectual property rights in some countries outside the United States can be less extensive than those in the United States and we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products or technology and may export otherwise infringing products or technology to territories where we have patent protection, but enforcement rights are not as strong as those in the United States. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. Further, the legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to pharmaceuticals or biologics, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any such lawsuits that we initiate and the damages and other remedies awarded, if any, may not be commercially meaningful.

Similarly, if our trade secrets are disclosed in a foreign jurisdiction, competitors worldwide could have access to our proprietary information and we may be without satisfactory recourse. Such disclosure could have a material adverse effect on our business. Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. In addition, certain developing countries, including China and India, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, we and our licensors may have limited remedies if patents are infringed or if we or our licensors are compelled to grant a license to a third-party, which could materially diminish the value of those patents. In addition, many countries limit the enforceability of patents against government agencies or government contractors. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Changes in patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs, and may diminish our ability to protect our inventions, obtain, maintain, and enforce our intellectual property rights and, more generally, could affect the value of our intellectual property or narrow the scope of our owned and licensed patents. Recent patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act, or the Leahy-Smith Act, signed into law on September 16, 2011, could increase those uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. After March 2013, under the Leahy-Smith Act, the United States transitioned to a first inventor to file system in which, assuming that the other statutory requirements are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third-party was the first to invent the claimed invention. A third party that files a patent application in the USPTO after March 2013, but before we file an application covering the same invention, could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (1) file any patent application related to our product candidates and other proprietary technologies we may develop or (2) invent any of the inventions claimed in our or our licensor's patents or patent applications. Even where we have a valid and enforceable patent, we may not be able to exclude others from practicing the claimed invention where the other party can show that they used the invention in commerce before our filing date or the other party benefits from a compulsory license. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, the patent positions of companies in the development and commercialization of pharmaceuticals are particularly uncertain. The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the 2013 case *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.*, the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of the patents owned or licensed by us will be found invalid based on this decision, we cannot predict how future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submissions, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuities fees and various other governmental fees on patents and/or patent applications are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent and/or patent application. The USPTO and various foreign governmental patent agencies also require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse, including due to the effect of the COVID-19 pandemic on us or our patent maintenance vendors, can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

We may rely on trade secret and proprietary know-how which can be difficult to trace and enforce and, if we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our technology and product candidates, we may also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. Elements of our product candidate, including processes for their preparation and manufacture, may involve proprietary know-how, information, or technology that is not covered by patents, and thus for these aspects we may consider trade secrets and know-how to be our primary intellectual property. Any disclosure, either intentional or unintentional, by our employees, the employees of third parties with whom we share our facilities or third-party consultants and vendors that we engage to perform research, clinical trials or manufacturing activities, or misappropriation by third parties (such as through a cybersecurity breach) of our trade secrets or proprietary information could enable competitors to duplicate or surpass our technological achievements, thus eroding our competitive position in our market. Because we expect to rely on third parties in the development and manufacture of our product candidates, we must, at times, share trade secrets with them. Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Trade secrets and know-how can be difficult to protect. We require our employees to enter into written employment agreements containing provisions of confidentiality and obligations to assign to us any inventions generated in the course of their employment. We and any third parties with whom we share facilities enter into written agreements that include confidentiality and intellectual property obligations to protect each party's property, potential trade secrets, proprietary know-how, and information. We further seek to protect our potential trade secrets, proprietary know-how, and information in part, by entering into non-disclosure and confidentiality agreements with parties who are given access to them, such as our corporate collaborators, outside scientific collaborators, contract research organizations, contract manufacturers, consultants, advisors and other third parties. With our consultants, contractors, and outside scientific collaborators, these agreements typically include invention assignment obligations. We cannot guarantee that we have entered into such agreements with each party that may have or has had access to our trade secrets or proprietary technology and processes. We cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third-party, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor or other third-party, our competitive position would be harmed.

We may become subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may be subject to claims that former employees, collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing our product candidates or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Our licensors may have relied on third-party consultants or collaborators or on funds from third parties, such as the U.S. government, such that our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights or other rights to our in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patent rights are of limited duration. In the United States, if all maintenance fees are paid timely, the natural expiration of a patent is generally 20 years after its first effective filing date. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such product candidates are commercialized. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from biosimilar or generic products. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing product candidates similar or identical to ours. Upon issuance in the United States, a patent's life can be increased based on certain delays caused by the USPTO, but this increase can be reduced or eliminated based on certain delays caused by the patent applicant during patent prosecution. A patent term extension based on regulatory delay may be available in the United States. However, only a single patent can be extended for each marketing approval, and any patent can be extended only once, for a single product. Moreover, the scope of protection during the period of the patent term extension does not extend to the full scope of the claim, but instead only to the scope of the product as approved. Laws governing analogous patent term extensions in foreign jurisdictions vary widely, as do laws governing the ability to obtain multiple patents from a single patent family. Additionally, we may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain approval of competing products following our patent expiration and may take advantage of our investment in development and clinical trials by referencing our

clinical and preclinical data to launch their product earlier than might otherwise be the case, and our revenue could be reduced, possibly materially.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our current or future trademarks or trade names may be challenged, infringed, circumvented or declared generic or descriptive, or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. During trademark registration proceedings, we may receive rejections of our applications by the USPTO or in other foreign jurisdictions. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names to third parties, such as distributors. Though these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names.

Moreover, any name we have proposed to use with our product candidate in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. Similar requirements exist in Europe. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA (or an equivalent administrative body in a foreign jurisdiction) objects to any of our proposed proprietary product names, it may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. Furthermore, in many countries, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. If we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Risks Related to Our Common Stock

The market price of our common stock has been and may continue to be volatile or may decline regardless of our operating performance and you could lose all or part of your investment.

The market price of our common stock may fluctuate significantly in response to numerous factors, many of which are beyond our control, including:

- overall performance of the equity markets;
- our operating performance and the performance of other similar companies;
- the published opinions and third-party valuations by banking and market analysts;
- results from our ongoing clinical trials and future clinical trials with our current and future product candidates or of our competitors;
- changes in our projected operating results that we provide to the public, our failure to meet these projections or changes in recommendations by securities analysts that elect to follow our common stock;
- regulatory or legal developments in the United States and other countries;

- changes in the structure of healthcare payment systems;
- the level of expenses related to future product candidates or clinical development programs;
- our failure to achieve product development goals in the timeframe we announce;
- announcements of acquisitions, strategic alliances or significant agreements by us or by our competitors;
- recruitment or departure of key personnel;
- the economy as a whole and market conditions in our industry;
- the expiration of market standoff or contractual lock-up agreements;
- the size of our market float;
- the ongoing and future impact of the COVID-19 pandemic and actions taken to slow its spread; and
- any other factors discussed in this Annual Report on Form 10-K.

In addition, the stock markets in general, and the Nasdaq Global Market in particular, have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many immuno-oncology companies. Stock prices of many immuno-oncology companies have fluctuated in a manner unrelated or disproportionate to the operating performance of those companies, and we have in the past experienced volatility that has been unrelated or disproportionate to our operating performance. From July 10, 2020 through March 5, 2021, the closing price of our common stock has ranged between \$7.63 and \$17.62 per share. The trading prices for common stock of other biopharmaceutical companies have also been highly volatile as a result of the COVID-19 pandemic. In the past, stockholders have filed securities class action litigation following periods of market volatility. If we were to become involved in securities litigation, it could subject us to substantial costs, divert resources and the attention of management from our business and adversely affect our business.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

As of March 5, 2021, our executive officers, directors, five percent stockholders and their affiliates beneficially owned approximately 69% of our voting stock. Therefore, these stockholders have the ability to influence us through their ownership positions. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

In addition, Dr. Ostertag, our Chief Executive Officer, a member of our board of directors and the beneficial owner of approximately 16% of our voting stock as of March 5, 2021, is the sole director of Demeetra AgBio, Inc., or Demeetra, serves as its President and Secretary and together with his affiliated entities, owns on a fully-diluted basis, 63% of its capital stock. Further, Dr. Ostertag is also a member of the board of directors of Hera Testing Laboratories, Inc., or Hera, and served as its Chief Executive Officer from inception until July 2015 and, together with his affiliated entities, owns on a fully-diluted basis, 42% of its capital stock.

As a result, the interests of Dr. Ostertag may not be aligned with the interests of you and our other stockholders with respect to our relationships with Hera and Demeetra, and he may from time to time be incentivized to take certain actions that benefit his interests in those other entities and that you and our other stockholders do not view as being in your interest as investors in our company.

If we fail to maintain an effective system of internal controls in the future, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act and the rules and regulations of the Nasdaq Stock Market. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal controls over financial reporting. Prior to the completion of our IPO, we were a private company with limited accounting personnel to adequately execute our accounting processes and other supervisory resources with which to address our internal control over financial reporting and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner.

We previously identified material weaknesses in our internal control over financial reporting. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim consolidated financial statements will not be prevented or detected on a timely basis. These material weaknesses related to a lack of a sufficient complement of accounting resources, which led to our inability to maintain segregation of duties between the creation and posting of journal entries and review of account reconciliations. While these material weaknesses did not result in a misstatement to our consolidated financial statements, these material weaknesses could have resulted in a misstatement of our accounts or disclosures that would result in a material misstatement to our annual or interim consolidated financial statements that would not be prevented or detected.

As discussed in Part II, Item 9A of this Report, we concluded that the material weaknesses previously identified were remediated as of December 31, 2020.

We may discover additional weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our consolidated financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls over financial reporting, we may not be able to produce timely and accurate financial statements. If that were to happen, our investors could lose confidence in our reported financial information, the market price of our stock could decline, and we could be subject to sanctions or investigations by the SEC or other regulatory authorities.

General Risk Factors

We will continue to incur significantly increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we have incurred and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, we are subject to the Sarbanes-Oxley Act, as well as rules subsequently implemented by the SEC, and various requirements the Nasdaq Global Select Market have imposed on public companies. In July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as "say on pay" and proxy access. Recent legislation permits smaller "emerging growth companies" to implement many of these requirements over a longer period and up to five years from the completion of our IPO. We intend to continue to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costlier. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to

maintain our current levels of such coverage. We estimate that we will annually incur approximately \$3.0 million to \$4.0 million in additional expenses to comply with the requirements imposed on us as a public company.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, contractual damages, reputational harm, diminished profits and future earnings, additional reporting obligations and oversight if subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations.

Our internal information technology systems, or those of our third-party CROs or other contractors or consultants, may fail or suffer security breaches, loss or leakage of data, and other disruptions, which could result in a material disruption of our product candidates' development programs, compromise sensitive information related to our business or prevent us from accessing critical information, potentially exposing us to liability or otherwise adversely affecting our business.

We are increasingly dependent upon information technology systems, infrastructure and data to operate our business. In the ordinary course of business, we collect, store and transmit confidential information (including but not limited to intellectual property, proprietary business information and personal information). It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such confidential information. We also have outsourced elements of our operations to third parties, and as a result we manage a number of third-party contractors who have access to our confidential information.

Despite the implementation of security measures, given their size and complexity and the increasing amounts of confidential information that they maintain, our internal information technology systems and those of our third-party CROs and other contractors and consultants are potentially vulnerable to breakdown or other damage or interruption from service interruptions, system malfunction, natural disasters, terrorism, war and telecommunication and electrical failures, as well as security breaches from inadvertent or intentional actions by our employees, contractors, consultants, business partners, and/or other third parties, or from cyber-attacks by malicious third parties (including the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information), which may compromise our system infrastructure or lead to data leakage. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and reputational damage and the further development and commercialization of our product candidates could be delayed.

While we have not experienced any such system failure, accident or security breach to date, we cannot assure you that our data protection efforts and our investment in information technology will prevent significant breakdowns, data leakages, breaches in our systems or other cyber incidents that could have a material adverse effect upon our reputation, business, operations or financial condition. For example, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs and the development of our product candidates could be delayed. In addition, the loss of clinical trial data for our product

candidates could result in delays in our marketing approval efforts and significantly increase our costs to recover or reproduce the data. Furthermore, significant disruptions of our internal information technology systems or security breaches could result in the loss, misappropriation, and/or unauthorized access, use, or disclosure of, or the prevention of access to, confidential information (including trade secrets or other intellectual property, proprietary business information, and personal information), which could result in financial, legal, business, and reputational harm to us. For example, any such event that leads to unauthorized access, use, or disclosure of personal information, including personal information regarding our clinical trial subjects or employees, could harm our reputation directly, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information, which could result in significant legal and financial exposure and reputational damages that could potentially have an adverse effect on our business.

Changes in tax laws or regulations that are applied adversely to us or our customers may have a material adverse effect on our business, cash flow, financial condition or results of operations.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could adversely affect our business operations and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, the Tax Act enacted many significant changes to the U.S. tax laws. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. For example, the CARES Act modified certain provisions of the Tax Act. In addition, it is uncertain if and to what extent various states will conform to the Tax Act or any newly enacted federal tax legislation. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses under the Tax Act or future reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

Failure to comply with health and data protection laws and regulations could lead to government enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business.

We and any potential collaborators may be subject to federal, state, and foreign data protection laws and regulations (i.e., laws and regulations that address privacy and data security). In the United States, numerous federal and state laws and regulations, including federal health information privacy laws, state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), that govern the collection, use, disclosure, and protection of health-related and other personal information could apply to our operations or the operations of our collaborators. In addition, we may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH. If we violate HIPAA, we may be subject to significant civil penalties. Additionally, depending on the facts and circumstances, we could be subject to significant penalties if we violate HIPAA.

Internationally, virtually every jurisdiction in which we operate has established its own data security and privacy legal framework that may also apply to health-related and other personal information obtained outside of the United States, including but not limited to the European Union, or EU. The unstable nature of EU's data protection landscape may result in possible significant operational costs for internal compliance and risk to our business. While we could take steps to mitigate the impact on us, such as implementing standard contractual clauses and self-certifying under the EU-US Privacy Shield, the efficacy and longevity of these mechanisms remains uncertain. In addition, the EU has adopted the General Data Protection Regulation, or GDPR, which went into effect on May 25, 2018 and contains numerous requirements and changes from existing EU law, including more robust obligations on data controllers and data processors, and heavier documentation requirements for data protection compliance programs by companies. Specifically, the GDPR contains numerous privacy-related changes for companies operating in the EU, including greater control for data subjects (e.g., the "right to be forgotten"), increased data portability for EU consumers, data breach notification requirements, and increased fines. In particular, under the GDPR, fines of up to 20 million euros or up to 4% of the annual global revenue of the noncompliant company, whichever is greater, could be imposed for violations of certain of the GDPR's requirements. The GDPR

requirements would apply not only to third-party transactions, but also to transfers of information between us and our subsidiaries, including employee information.

Compliance with the GDPR may cause us to incur substantial operational costs or require us to change our business practices. Despite our efforts to bring practices into compliance before the effective date of the GDPR, we may not be successful either due to internal or external factors such as resource allocation limitations or a lack of vendor cooperation. Non-compliance could result in proceedings against us by governmental entities, customers, data subjects, or others. We may also experience difficulty retaining or obtaining new European or multi-national customers due to the legal requirements, compliance cost, potential risk exposure, and uncertainty for these entities, and we may experience significantly increased liability with respect to these customers pursuant to the terms set forth in our engagements with them. We may find it necessary to establish systems to maintain personal data originating from the EU in the European Economic Area, which may involve substantial expense and distraction from other aspects of our business. In the meantime, there could be uncertainty as to how to comply with EU privacy law. Separately, the United Kingdom's withdrawal from the EU could also lead to further legislative and regulatory changes and increase our compliance costs. In particular, the United Kingdom has transposed the GDPR into domestic law with a UK version of the GDPR taking effect from January 2021 (after the end of the transitional period) which could expose us to two parallel regimes each with the power to fine up to the greater of either 4% of global revenue, or Euro 20,000,000 (for the EU) or £17,500,000 (for the UK).

Failure to comply with U.S. and international data protection laws and regulations could result in government enforcement actions (which could include civil or criminal penalties), private litigation, breach reporting requirements and/or adverse publicity and could negatively affect our operating results and business. Moreover, clinical trial subjects about whom we or our potential collaborators obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations. We can face serious consequences for violations.

U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations, or collectively, Trade Laws, prohibit, among other things, companies and their employees, agents, CROs, legal counsel, accountants, consultants, contractors, and other partners from authorizing, promising, offering, providing, soliciting, or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of Trade Laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We also expect our non-U.S. activities to increase over time. We expect to rely on third parties for research, preclinical studies, and clinical trials and/or to obtain necessary permits, licenses, patent registrations, and other marketing approvals. We can be held liable for the corrupt or other illegal activities of our personnel, agents, or partners, even if we do not explicitly authorize or have prior knowledge of such activities.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We, and the third parties with whom we share our facilities, are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Each of our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. Each of our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. We could be held liable for any resulting damages in the event of contamination or injury resulting from the use of hazardous materials by us or the third parties with whom we share our facilities, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research and development. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and stock price.

As a result of the COVID-19 pandemic and actions taken to slow its spread, the global credit and financial markets have recently experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy has been and may continue to be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive an economic downturn, which could directly affect our ability to attain our operating goals on schedule and on budget.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

Additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner, we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

Pursuant to our 2020 Equity Incentive Plan, or the 2020 Plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. The number of shares of our common stock reserved for issuance under our 2020 Plan will automatically increase on January 1 of each calendar year, starting on January 1, 2021 through January 1, 2030, in an amount equal to the lesser of (i) 5% of the total number of shares of our common stock outstanding on the last day of the calendar month before the date of each automatic increase, or (ii) a lesser number of shares determined by our board of directors prior to the applicable January 1st. If our board of directors elects to increase the number of shares available for future grant by the maximum amount each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

If securities or industry analysts issue an adverse or misleading opinion regarding our stock, our stock price and trading volume could decline.

The trading market for our common stock could be influenced by the research and reports that industry or securities analysts publish about us or our business. If any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our stock performance, or if the clinical trials and operating results fail to meet the expectations of analysts, the trading price for our common stock would be negatively affected. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, demand for our common stock could decrease, which might cause our common stock price and trading volume to decline.

Requirements associated with being a public company will increase our costs significantly, as well as divert significant company resources and management attention.

We are subject to the reporting requirements of the Exchange Act, or the other rules and regulations of the SEC or any securities exchange relating to public companies. Compliance with the various reporting and other requirements applicable to public companies requires considerable time and attention of management. We cannot assure you that we will satisfy our obligations as a public company on a timely basis.

In addition, as a public company, it may be more difficult or more costly for us to obtain certain types of insurance, including directors' and officers' liability insurance, and we may be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified personnel to serve on our board of directors, our board committees or as executive officers.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

We are subject to the periodic reporting requirements of the Exchange Act. We designed our disclosure controls and procedures to reasonably assure that information we must disclose in reports we file or submit under the Exchange Act is accumulated and communicated to management, and recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well-conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. For example, our directors or executive officers could inadvertently fail to disclose a new relationship or arrangement causing us to fail to make any related party transaction disclosures. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements due to error or fraud may occur and not be detected.

Future changes in financial accounting standards or practices may cause adverse and unexpected revenue fluctuations and adversely affect our reported results of operations.

Future changes in financial accounting standards may cause adverse, unexpected revenue fluctuations and affect our reported financial position or results of operations. Financial accounting standards in the United States are constantly under review and new pronouncements and varying interpretations of pronouncements have occurred with frequency in the past and are expected to occur again in the future. As a result, we may be required to make changes in our accounting policies. Those changes could affect our financial condition and results of operations or the way in which such financial condition and results of operations are reported. We intend to invest resources to comply with evolving standards, and this investment may result in increased general and administrative expenses and a diversion of management time and attention from business activities to compliance activities. See the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations—Recent Accounting Pronouncements."

We are an "emerging growth company," and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an “emerging growth company” as defined in the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- not being required to hold a non-binding advisory vote on executive compensation or obtain stockholder approval of any golden parachute payments not previously approved.

In addition, as an “emerging growth company” the JOBS Act allows us to delay adoption of new or revised accounting pronouncements applicable to public companies until such pronouncements are made applicable to private companies, unless we later irrevocably elect not to avail ourselves of this exemption. We have elected to use this extended transition period under the JOBS Act. As a result, our consolidated financial statements may not be comparable to the financial statements of issuers who are required to comply with the effective dates for new or revised accounting standards that are applicable to public companies, which may make comparison of our financials to those of other public companies more difficult.

We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of our IPO, (b) in which we have total annual gross revenue of at least \$1.07 billion or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the prior June 30th and (2) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

Delaware law and provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make a merger, tender offer or proxy contest difficult, thereby depressing the trading price of our common stock.

Our status as a Delaware corporation and the anti-takeover provisions of the Delaware General Corporation Law may discourage, delay or prevent a change in control by prohibiting us from engaging in a business combination with an interested stockholder for a period of three years after the person becomes an interested stockholder, even if a change of control would be beneficial to our existing stockholders. In addition, our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that may make the acquisition of our company more difficult, including the following:

- a classified board of directors with three-year staggered terms, which could delay the ability of stockholders to change the membership of a majority of our board of directors;
- the ability of our board of directors to issue shares of preferred stock and to determine the price and other terms of those shares, including preferences and voting rights, without stockholder approval, which could be used to significantly dilute the ownership of a hostile acquirer;
- the exclusive right of our board of directors to elect a director to fill a vacancy created by the expansion of our board of directors or the resignation, death or removal of a director, which prevents stockholders from being able to fill vacancies on our board of directors;

- a prohibition on stockholder action by written consent, which forces stockholder action to be taken at an annual or special meeting of our stockholders;
- the requirement that a special meeting of stockholders may be called only by a majority vote of our entire board of directors, the chairman of our board of directors or our Chief Executive Officer, which could delay the ability of our stockholders to force consideration of a proposal or to take action, including the removal of directors;
- the requirement for the affirmative vote of holders of at least 66-2/3% of the voting power of all of the then-outstanding shares of the voting stock, voting together as a single class, to amend the provisions of our amended and restated certificate of incorporation relating to the management of our business or our amended and restated bylaws, which may inhibit the ability of an acquirer to affect such amendments to facilitate an unsolicited takeover attempt; and
- advance notice procedures with which stockholders must comply to nominate candidates to our board of directors or to propose matters to be acted upon at a stockholders' meeting, which may discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of us.

In addition, as a Delaware corporation, we are subject to Section 203 of the Delaware General Corporation Law. These provisions may prohibit large stockholders, in particular those owning 15% or more of our outstanding voting stock, from merging or combining with us for a certain period of time. A Delaware corporation may opt out of this provision by express provision in its original certificate of incorporation or by amendment to its certificate of incorporation or bylaws approved by its stockholders. However, we have not opted out of this provision.

These and other provisions in our amended and restated certificate of incorporation, amended and restated bylaws and Delaware law could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by our then-current board of directors, including delay or impede a merger, tender offer or proxy contest involving our company. The existence of these provisions could negatively affect the price of our common stock and limit opportunities for you to realize value in a corporate transaction.

Our amended and restated certificate of incorporation designates the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against our company and our directors, officers and employees.

Our amended and restated certificate of incorporation provides that, to the fullest extent permitted by law, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (1) any derivative action or proceeding brought on our behalf; (2) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders; (3) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws; (4) any action or proceeding to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws; (5) any action or proceeding as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and (6) any action asserting a claim against us or any of our directors, officers or other employees, governed by the internal affairs doctrine.

This provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation further provides that the federal district courts of the United States of America will be the exclusive forum for resolving any

complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find either exclusive forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

Item 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

We currently lease approximately 83,000 square feet of manufacturing, office and laboratory space in San Diego, California under a lease that expires on December 31, 2029, which includes a pilot manufacturing facility adjacent to our office and lab space. We believe our existing leased space is sufficient to meet our facilities needs for the foreseeable future and that any additional space we may require will be available on commercially reasonable terms.

Item 3. Legal Proceedings.

We are not currently a party to any material legal proceedings. From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. Regardless of outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, negative publicity, reputational harm and other factors.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.**Market Information**

Our common stock began trading on The Nasdaq Global Select Market on July 10, 2020 and trades under the symbol “PSTX.” Prior to July 10, 2020, there was no public market for our common stock.

Holders of Record

As of March 5, 2021, there were approximately 128 stockholders of record of our common stock. Certain shares are held in “street” name and accordingly, the number of beneficial owners of such shares is not known or included in the foregoing number.

Dividend Policy

We have never declared or paid any dividends on our common stock. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors, subject to applicable laws and will depend upon, among other factors, our results of operations, financial condition, contractual restrictions and capital requirements. In addition, our loan agreement with Oxford governing our indebtedness contains restrictions on our ability to declare and pay cash dividends on our capital stock.

Securities Authorized for Issuance under Equity Compensation Plans

The information called for by this item is incorporated by reference to our definitive proxy statement for the 2021 Annual Meeting of Stockholders. See Part III, Item 12 “Security Ownership of Certain Beneficial Owners and Management.”

Recent Sales of Unregistered Securities

From January 1, 2020 through July 8, 2020, which is the day before we priced our IPO, we granted to our directors, employees, and consultants options to purchase 447,071 shares of common stock under our 2015 Equity Incentive Plan, as amended, or the 2015 Plan, with a weighted-average per share exercise price of \$12.25. Of such options, none were exercised as of December 31, 2020. These transactions were exempt from the registration requirements of the Securities Act in reliance upon Rule 701 promulgated under the Securities Act of 1933, as amended.

In June 2020, we sold an aggregate of 10,018,300 shares of our Series D preferred stock and received gross proceeds of approximately \$109.5 million. This transaction was exempt from the registration requirements of the Securities Act in reliance upon Section 4(a)(2) of the Securities Act or Regulation D promulgated under the Securities Act.

Use of Proceeds

We completed our IPO pursuant to a Registration Statement on Form S-1 (File No. 333-239321) that was declared effective on July 9, 2020 and registered an aggregate of 16,100,000 shares of our common stock. On July 14, 2020, we sold 14,000,000 shares of our common stock at a public offering price of \$16.00 per share for an aggregate gross offering price of \$224.0 million.

The net proceeds to us after deducting underwriting discounts and commissions of \$15.7 million and net offering expenses of \$2.6 million were \$205.7 million. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

Upon receipt, the net proceeds from our IPO were held in cash and cash equivalents and short-term investments, primarily bank money market accounts. Through March 5, 2021, we have not used any of the net proceeds from our IPO. We are investing these funds in a combination of short- and intermediate-term, interest-bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government. There has been no material change in the planned use of proceeds from our IPO from those disclosed in the Registration Statement.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

None.

Item 6. Selected Consolidated Financial Data.

Not applicable.

Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K. This discussion, particularly information with respect to our future results of operations or financial condition, business strategy and plans and objectives of management for future operations, includes forward-looking statements that involve risks and uncertainties as described under the heading “Forward-Looking Statements” in this Annual Report on Form 10-K. You should review the disclosure under the heading “Risk Factors” in this Annual Report on Form 10-K for a discussion of important factors that could cause our actual results to differ materially from those anticipated in these forward-looking statements.

Overview

We are a clinical-stage biopharmaceutical company dedicated to utilizing our proprietary gene engineering platform technologies to create next generation cell and gene therapeutics with the capacity to cure. We have discovered and are developing a broad portfolio of product candidates in a variety of indications based on our core proprietary platform, including our non-viral piggyBac DNA Delivery System, Cas-CLOVER Site-Specific Gene Editing System and nanoparticle- and AAV-based gene delivery technologies. Our core platform technologies have utility, either alone or in combination, across many cell and gene therapeutic modalities and enable us to engineer our wholly-owned portfolio of product candidates that are designed to overcome the primary limitations of current generation cell and gene therapeutics.

Within cell therapy, we believe our technologies allow us to create product candidates with engineered cells that engraft in the patient’s body and drive lasting durable responses that may have the capacity to result in single treatment cures. Our CAR-T therapy portfolio consists of both autologous and allogeneic, or off-the-shelf, product candidates. We are advancing a broad pipeline with a plan to have multiple CAR-T product candidates in the clinic in 2021 in both hematological and solid tumor oncology indications. Within gene therapy, we believe our technologies have the potential to create next generation therapies that can deliver long-term, stable gene expression that does not diminish over time and that may have the capacity to result in single treatment cures. The following table summarizes our current product candidate portfolio:

Indication	Candidate	Preclinical	IND-Enabling	Phase 1	Phase 2	Phase 3
CAR-T FOR ONCOLOGY						
MULTIPLE MYELOMA	P-BCMA-101	Auto				
	P-BCMA-ALLO1	Allo				
	Dual CAR (BCMA/CD19)	Allo				
PROSTATE CANCER	P-PSMA-101	Auto				
	P-PSMA-ALLO1	Allo				
SOLID TUMOR	P-MUC1C-ALLO1	Allo				
	Dual CAR (Undisclosed)	Allo				
B - CELL	Dual CAR (CD19/CD20)	Allo				
LIVER DIRECTED GENE THERAPIES						
ORNITHINE TRANSCARBAMYLASE DEFICIENCY (OTC)	P-OTC-101	GT				
HEMOPHILIA A	P-FVIII-101	GT				

We were incorporated in December 2014 and subsequently spun out from Transposagen, a company that has been developing gene engineering technologies since 2003. Since our inception, our operations have focused on organizing and staffing our company, business planning, raising capital, in-licensing, developing and acquiring intellectual property rights and establishing and protecting our intellectual property portfolio, developing our gene engineering technologies, identifying potential product candidates and undertaking research and development and manufacturing activities, including preclinical studies and clinical trials of our product candidates, and engaging in

strategic transactions. We do not have any product candidates approved for sale and have not generated any revenue from product sales. We have funded our operations primarily through the sale of equity. Since our inception, we have raised an aggregate of \$334.3 million of gross proceeds from the sale of shares of our redeemable convertible preferred stock, received \$30.0 million of gross proceeds from borrowings under our loan agreement and received an aggregate of \$23.8 million in grant funding from the California Institute of Regenerative Medicine, or CIRM. On July 14, 2020, we completed our IPO pursuant to which we issued and sold 14,000,000 shares of common stock for gross proceeds of \$224.0 million. As of December 31, 2020, we had cash, cash equivalents and short-term investments of \$309.2 million. Since our inception, we have incurred significant operating losses and expect to continue to incur significant operating losses for the foreseeable future. Our net losses were \$129.8 million and \$86.5 million for the years ended December 31, 2020 and 2019, respectively. As of December 31, 2020, we had an accumulated deficit of \$281.9 million.

We expect our expenses and losses to increase substantially for the foreseeable future as we continue our development of, and seek regulatory approvals for, our product candidates, including P-BCMA-101 and P-PSMA-101, and begin to commercialize any approved products, as well as hire additional personnel, pay for accounting, audit, legal, regulatory and consulting services, and pay costs associated with maintaining compliance with Nasdaq listing rules and SEC requirements, director and officer insurance, investor and public relations activities and other expenses associated with operating as a public company. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities.

We do not expect to generate any revenues from product sales unless and until we successfully complete development and obtain regulatory approval for P-BCMA-101, P-PSMA-101, or any other product candidates, which will not be for at least the next several years, if ever. If we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Accordingly, until such time, if ever, as we can generate substantial product revenue, we expect to finance our operations through equity offerings, debt financings or other capital sources, including potentially grants, collaborations, licenses or other similar arrangements. However, we may not be able to secure additional financing or enter into such other arrangements in a timely manner or on favorable terms, if at all. Especially in light of the COVID-19 pandemic, we can give no assurances that we will be able to secure such additional sources of funds to support our operations, or, if such funds are available to us, that such additional financing will be sufficient to meet our needs. Our failure to raise capital or enter into such other arrangements when needed would have a negative impact on our financial condition and could force us to delay, reduce or terminate our research and development programs or other operations, or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

The manufacturing process for our allogeneic product candidates is nearly identical to the process for our autologous product candidates, except for the gene editing and related steps. We work with a number of third-party contract manufacturers for production of our product candidates. We also work with a variety of suppliers to provide our manufacturing raw materials including media, DNA and RNA components. We have completed construction of an internal pilot GMP manufacturing facility in San Diego adjacent to our headquarters to develop and manufacture preclinical materials and clinical supplies of our product candidates for Phase 1 and Phase 2 clinical trials in the future. We commenced operations in this facility in the second half of 2020, but we expect that we will continue to rely on third parties for various manufacturing needs. In the future, we may also build one or more commercial manufacturing facilities for any approved product candidates.

License Agreements

Below is a summary of the key terms for certain of our license agreements. For a more detailed description of these and our other license agreements, see the section titled “Business—License Agreements” and Note 10 to our annual consolidated financial statements.

License Agreement with Janssen Biotech Inc.

On August 3, 2015, we entered into a license agreement with Janssen, or the Janssen Agreement, pursuant to which we obtained exclusive worldwide rights to research, develop, manufacture and commercialize pharmaceutical

products comprising autologous CAR-modified T-cells or any CAR-modified natural killer or CAR-modified natural-killer-like cells expressing certain Centyrin molecules CAR-modified for the treatment or prevention of any disease in humans. This is the binding technology we use in our P-BCMA-101 and P-PSMA-101 product candidates. Under the Janssen Agreement, we also have the right to screen Janssen's Centyrin library for agents that bind or modify targets of interest for our internal research and development purposes for potential use in a licensed product.

Pursuant to the Janssen Agreement, we paid Janssen an upfront fee of \$0.2 million. As of December 31, 2020, we have paid approximately \$3.3 million in milestone development fees relating to P-BCMA-101 and approximately \$0.7 million in milestone development fees relating to P-PSMA-101. We are required to pay Janssen up to an aggregate of \$75.8 million upon the achievement of certain clinical, regulatory and sales milestones for the first licensed product and up to an aggregate of \$46.8 million upon the achievement of certain clinical, regulatory and sales milestones for each licensed product thereafter. We are also obligated to pay, on a product-by-product and country-by-country basis, royalties in the low single-digit percentage range on annual net sales, with the royalty rates varying depending on if there is a valid claim present within the licensed patent rights covering the licensed product in the applicable country in which the net sales occur. The royalty rates are subject to reduction upon certain events.

April 2017 Commercial License Agreement with TeneoBio, Inc.

On April 27, 2017, we entered into a commercial license agreement with TeneoBio, or the 2017 TeneoBio Agreement, pursuant to which we obtained exclusive worldwide rights to use and develop pharmaceutical products comprising allogeneic T-cells expressing a CAR molecule containing certain heavy chain sequences provided by TeneoBio for the treatment of human disease. We use this heavy-chain-only binder in our P-BCMA-ALLO1 product candidate.

Pursuant to the 2017 TeneoBio Agreement, we have paid TeneoBio \$0.5 million through our selection of the antibodies licensed under the 2017 TeneoBio Agreement. We are required to pay TeneoBio up to an aggregate of \$20.5 million upon the first achievement of certain clinical and regulatory milestones for any allogeneic product and up to an aggregate of \$20.5 million upon the first achievement of certain clinical and regulatory milestones for any autologous product. We are also obligated to pay, on a product-by-product and country-by-country basis, a royalty in the low single-digit percentage on net sales of any licensed products.

August 2018 Commercial License Agreement with TeneoBio, Inc.

On August 3, 2018, we entered into a commercial license agreement, or the 2018 TeneoBio Agreement, with TeneoBio for the development and use of TeneoBio's human heavy-chain-only antibodies in CAR-T cell therapies. Under the terms of the 2018 TeneoBio Agreement, we have the option to obtain exclusive rights to research, develop and commercialize up to a certain number of targets from TeneoBio.

Pursuant to the 2018 TeneoBio Agreement, we paid TeneoBio an upfront fee of \$4.0 million. We are required to pay additional fees in the low- to mid-six figure dollar range upon (1) selecting exclusivity for a particular target, which restricts TeneoBio from licensing that particular target to a third party for a period of time, (2) continuing exclusivity for any selected target on each anniversary thereafter and (3) exercising our commercial option for each target. We are required to pay TeneoBio up to an aggregate of \$31.0 million upon the first achievement of certain clinical and regulatory milestones for each licensed product. We are also obligated to pay, on a product-by-product and country-by-country basis, a low single-digit percentage royalty on net sales of any licensed products. The royalty rate is subject to reduction upon certain events.

October 2019 License Agreement with Genus Oncology, LLC

On October 24, 2019, we entered into a license agreement with Genus, or the Genus Agreement. Pursuant to the Genus Agreement, we paid Genus an upfront fee of \$1.5 million and Genus granted us the option, which was exercised in April 2020 for an additional \$1.5 million fee, to obtain an exclusive worldwide license under certain patents and a non-exclusive worldwide license under certain know-how controlled by Genus to research, develop and commercialize pharmaceutical products incorporating CAR cells expressing antibodies and derivatives thereof targeting MUC1, or a Genus licensed product, and a non-exclusive worldwide license under certain patents and know-how controlled by Genus to research, develop and commercialize companion diagnostics for the treatment, prevention and palliation of human diseases and conditions. We may use a Genus antibody or derivative thereof targeting MUC1 as a binder in our P-MUC1C-ALLO1 product candidate.

Pursuant to the Genus Agreement, we are also required to pay Genus up to an aggregate of \$71.0 million upon first achievement of certain clinical, regulatory and sales milestones for any Genus licensed product and companion diagnostics. We are also obligated to pay, on a product-by-product and country-by-country basis, tiered royalties in the low to mid-single-digit percentage on net sales of any Genus licensed products and related companion diagnostics. The royalty rate is subject to reduction upon certain events.

Acquisition of Vindico

On October 10, 2016, we completed the acquisition of all the outstanding ownership interests in Vindico NanoBiotechnology, Inc., or Vindico, a company with expertise in polymer-based nanoparticle technology for delivery of, for example, gene therapy technologies. We paid \$1.1 million in cash and issued an aggregate of 350,522 shares of common stock to the selling shareholders. The common stock was valued at \$0.7 million based on the fair value of our common stock at October 10, 2016 or \$1.88 per share. We paid additional cash consideration of \$0.6 million in 2017.

In connection with the Vindico acquisition, we agreed to pay additional purchase consideration, based on the achievement of a certain developmental milestone using the acquired technology by October 2018, payable in shares of our common stock. In July 2018, we amended the terms of the Vindico merger agreement, which included an extension of contingency period through July 2019, the calculation to determine the number of shares to be settled and an option to settle the contingency in cash under certain circumstances. In July 2019, the developmental milestone was met and pursuant to the terms of the agreement, we subsequently issued 866,125 shares of common stock, valued at \$10.6 million to the former Vindico shareholders in August 2019. There is no further consideration due related to the Vindico acquisition.

CIRM Grant Funding

In December 2017, we were granted an award in the amount of \$19.8 million from CIRM to support our clinical trial for P-BCMA-101. The terms of the award include an option to repay the grant or convert it to a royalty obligation upon commercialization of the program. Based upon the terms of the agreement, we will record proceeds as a liability when received. As of December 31, 2020, proceeds received from the grant totaled \$19.7 million. We may receive up to \$0.1 million in future milestone payments.

In September 2018, we were granted an additional award in the amount of \$4.0 million from CIRM to support our preclinical studies for P-PSMA-101. As of December 31, 2020, all \$4.0 million had been received and no additional future payments were remaining.

Components of Our Results of Operations

Operating Expenses

Research and Development

Research and development expenses consist primarily of external and internal costs incurred for our research and development activities, including development of our platform technologies, our drug discovery efforts and the development of our product candidates.

External costs include:

- expenses incurred in connection with the preclinical and clinical development of our product candidates and research programs, including under agreements with third parties, such as consultants, contractors and contract research organizations;
- the cost of developing and scaling our manufacturing process and manufacturing drug products for use in our preclinical studies and clinical trials, including under agreements with third parties, such as consultants, contractors and contract manufacturing organizations, or CMOs;
- payments made under third-party licensing agreements; and
- laboratory supplies and research materials.

Internal costs include:

- personnel-related expenses, consisting of employee salaries, related benefits and stock-based compensation expense for employees engaged in research and development functions;
- facilities, depreciation and other expenses, consisting of direct and allocated expenses for rent and maintenance of facilities and insurance; and
- any impairment of indefinite-lived in process research and development, or IPR&D, related assets.

We expense research and development costs as incurred. External expenses are recognized based on an evaluation of the progress to completion of specific tasks using information provided to us by our service providers or our estimate of the level of service that has been performed at each reporting date. Upfront payments and milestone payments made for the licensing of technology are expensed as research and development in the period in which they are incurred. Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses or other long-term assets. These amounts are expensed as the related goods are delivered or the services are performed.

At any one time, we are working on multiple research programs. We track external costs by the stage of program, clinical or preclinical. Our internal resources, employees and infrastructure are not directly tied to any one program and are typically deployed across multiple programs. As such, we do not track internal costs on a specific program basis.

Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to CRO activity and manufacturing expenses. We expect that our research and development expenses will increase substantially in connection with our planned preclinical and clinical development activities in the near term and in the future, including in connection with our ongoing Phase 1 exploratory trial and Phase 2 trial of P-BCMA-101 for the treatment of patients with relapsed/refractory multiple myeloma and Phase 1 trial of P-PSMA-101 for the treatment of patients with mCRPC. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any of our product candidates. Our development costs may vary significantly based on factors such as:

- the number and scope of preclinical and IND-enabling studies;
- per patient trial costs;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the number of patients that participate in the trials;
- the drop-out or discontinuation rates of patients;

- potential additional safety monitoring requested by regulatory agencies;
- the duration of patient participation in the trials and follow-up;
- the cost and timing of manufacturing our product candidates;
- the phase of development of our product candidates;
- the efficacy and safety profile of our product candidates;
- the extent to which we establish additional licensing agreements; and
- whether we choose to partner any of our product candidates and the terms of such partnership.

A change in the outcome of any of these variables with respect to the development of any of our product candidates could significantly change the costs and timing associated with the development of that product candidate. We may never succeed in obtaining regulatory approval for any of our product candidates. We may obtain unexpected results from our clinical trials and preclinical studies.

General and Administrative

General and administrative expenses consist primarily of salaries and related costs, including stock-based compensation, for personnel in executive, finance and administrative functions. General and administrative expenses also include direct and allocated facility-related costs as well as professional fees for legal, patent, consulting, investor and public relations, accounting and audit services. We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research activities and development of our product candidates, including P-BCMA-101 and P-PSMA-101, and begin to commercialize any approved products. We also anticipate that our general and administrative expenses will increase as a result of payments for accounting, audit, legal, regulatory and consulting services, as well as costs associated with maintaining compliance with Nasdaq listing rules and SEC requirements, director and officer insurance, investor and public relations activities and other expenses associated with operating as a public company.

Increase (Decrease) in Contingent Consideration

In connection with our acquisition of Vindico in October 2016, we agreed to pay additional consideration based on the achievement of a certain developmental milestone using the acquired technology. The additional purchase consideration was payable in shares of our common stock. The number of shares of common stock issuable and the associated fair value could vary depending on (1) the price paid by investors in a qualified equity financing prior to the achievement of the milestone and (2) when and if the milestone is reached. We classified this contingent consideration as a liability on our consolidated balance sheets that was remeasured to fair value at each reporting date, and we recognized changes in the fair value of the contingent consideration liability as a component of operating expenses in our consolidated statements of operations and comprehensive loss. We recognized changes in the fair value of the contingent consideration liability until the milestone was met in July 2019. Upon issuance of the common stock related to the milestone in July 2019, the liability was reclassified to stockholder's deficit, within additional paid-in capital. For additional detail, see the subsections titled "—Acquisition of Vindico" above.

Other Income (Expense)

Interest Expense

Interest expense consists of (1) interest expense on outstanding borrowings under our loan agreement and (2) amortization of debt discount and debt issuance costs.

Other Income (Expense), Net

Other income (expense), net consists of (1) interest income and (2) miscellaneous income and expense unrelated to our core operations.

Interest income is comprised of interest earned on our invested cash balances in short-term investments. We expect our interest income to decrease based on available market interest rates.

Miscellaneous income and expense unrelated to our core operations is comprised of changes in fair value of warrant liability. We issued warrants to purchase shares of our Series A-1 preferred stock in connection with our loan agreement in July 2017. We issued additional warrants to purchase shares of our Series B preferred stock in connection with the amendment of our loan agreement in August 2018 and in February 2019. We classified these warrants as a liability on our consolidated balance sheets that we remeasured to fair value at each reporting date, and we recognized changes in the fair value of the warrant liability as a component of other income (expense), net in our consolidated statements of operations and comprehensive loss. We continued to recognize changes in the fair value of the warrant liability until the warrants qualified for equity classification. Upon the closing of the IPO, the preferred stock warrants became exercisable for common stock instead of preferred stock and the fair value of the warrant liability at the date of the IPO was reclassified to additional paid-in-capital. For additional detail, see the subsection titled “—Critical Accounting Policies and Significant Judgments and Estimates—Valuation of Warrants to Purchase Preferred Stock” below and Note 5 to our annual consolidated financial statements.

Results of Operations

Comparison of the Years Ended December 31, 2020 and 2019

The following table summarizes our results of operations for the years ended December 31, 2020 and 2019 (in thousands):

	Year Ended December 31,		Change
	2020	2019	
Operating expenses:			
Research and development	\$ 103,520	\$ 60,393	\$ 43,127
General and administrative	23,029	18,457	4,572
Increase in contingent consideration	—	6,683	(6,683)
Total operating expenses	126,549	85,533	41,016
Loss from operations	(126,549)	(85,533)	(41,016)
Other income (expense):			
Interest expense	(3,506)	(3,553)	47
Other income, net	280	2,559	(2,279)
Net loss before income tax	(129,775)	(86,527)	(43,248)
Income tax benefit	—	—	—
Net loss	\$ (129,775)	\$ (86,527)	\$ (43,248)

Research and Development Expenses

The following table summarizes our research and development expenses for the years ended December 31, 2020 and 2019 (in thousands):

	Year Ended December 31,		Change
	2020	2019	
External costs:			
Clinical stage programs ⁽¹⁾	\$ 41,085	\$ 27,441	\$ 13,644
Preclinical stage programs and other unallocated expenses	24,386	14,819	9,567
Internal costs:	—	—	—
Personnel	30,891	15,417	15,474
Facilities and other	7,158	2,716	4,442
Total research and development expenses	\$ 103,520	\$ 60,393	\$ 43,127

-
- (1) Clinical stage programs include costs related to P-BCMA-101 for the year ending December 31, 2019 and costs related to P-BCMA-101 and P-PSMA-101 for the year ending December 31, 2020.

Research and development expenses were \$103.5 million for the year ended December 31, 2020, compared to \$60.4 million for the year ended December 31, 2019. The increase in research and development expenses of \$43.1 million was primarily due to increases in the following: \$15.5 million of personnel expenses related to increased headcount, \$13.6 million of external costs related to our clinical stage programs including the ongoing enrollment and manufacturing for the P-BCMA-101 Phase 1 and Phase 2 clinical trial and initiation of the Phase 1 P-PSMA-101 trial, \$9.6 million of external costs related to our preclinical programs, and \$4.4 million of internal costs related to facilities and other expenses.

General and Administrative Expenses

General and administrative expenses were \$23.0 million for the year ended December 31, 2020, compared to \$18.5 million for the year ended December 31, 2019. The increase in general and administrative expenses of \$4.5 million was primarily due to an increase of \$5.9 million of personnel expenses related to increased headcount and a decrease of \$1.4 million of professional fees, largely due to the financing costs expensed in 2019 related to the IPO work completed prior to closing a private round of financing, offset by higher costs incurred in 2020 related to being a public company.

Increase in Contingent Consideration

Our contingent consideration liability relates to the Vindico acquisition, in which we had a contingent obligation to issue shares of our common stock to former Vindico shareholders upon achievement of a proof of concept preclinical milestone. Increase in contingent consideration was \$0 for the year ended December 31, 2020, compared to \$6.7 million for the year ended December 31, 2019. In the 2019 period, we recorded an increase in our contingent consideration liability resulting from the successful completion of the milestone in July 2019. Upon issuance of the common stock related to the milestone in July 2019, the liability was reclassified to stockholder's deficit, within additional paid-in capital.

Interest Expense

Interest expense was \$3.5 million for the year ended December 31, 2020, compared to \$3.6 million for the year ended December 31, 2019. Interest expense consisted of interest on the outstanding principal under our loan and security agreement, which was consistent during the respective periods.

Other Income, Net

Other income was \$0.3 million for the year ended December 31, 2020, compared to other income of \$2.6 million for the year ended December 31, 2019. This decrease in other income of \$2.3 million was primarily due to \$0.7 million of interest income partially offset by a \$0.4 million increase in warrant liability during the year ended December 31, 2020, compared to \$2.0 million of interest income and a \$0.5 million decrease of warrant liability during the year ended December 31, 2019. The decrease in interest income was driven by a decrease in available interest rates for investments.

Liquidity and Capital Resources

We were incorporated in December 2014 and subsequently spun out from Transposagen, a company that has been developing gene engineering technologies since 2003. Since our inception in 2014, we have incurred significant operating losses. Our net losses were \$129.8 million and \$86.5 million for the years ended December 31, 2020 and 2019, respectively. As of December 31, 2020, we had an accumulated deficit of \$281.9 million. Our operations have focused on organizing and staffing our company, business planning, raising capital, in-licensing and acquiring intellectual property rights and establishing and protecting our intellectual property portfolio, developing our gene engineering technologies, identifying potential product candidates and undertaking research and development and manufacturing activities, including preclinical studies and clinical trials of our product candidates,

and engaging in strategic transactions. Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures, and to a lesser extent, general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses. We have not yet commercialized any of our product candidates and we do not expect to generate revenue from sales of any product candidates for several years, if at all. We have funded our operations primarily through the sale of equity. Since our inception, we have raised \$224.0 million of gross proceeds from the sale of our common stock in our IPO, raised an aggregate of \$334.3 million of gross proceeds from the sale of shares of our redeemable convertible preferred stock, received \$30.0 million of gross proceeds from borrowings under our loan agreement and received an aggregate of \$23.8 million in grant funding from CIRM. We expect that our cash, cash equivalents and marketable securities as of December 31, 2020 of \$309.2 million will be sufficient to fund our operations for at least the next twelve months from the date of issuance of the financial statements included in this Annual Report on Form 10-K. In the long term we will need additional financing to support our continuing operations and pursue our growth strategy.

Loan Agreement

In July 2017, we entered into a loan and security agreement, or 2017 Loan Agreement, with Oxford. Under the original terms, the facility provided \$15.0 million, of which we drew \$10.0 million. In August 2018, we entered into an amended and restated agreement with Oxford, or the 2018 Loan Agreement, to, among other things, increase the size of the facility to \$30.0 million, modify the interest rate and extend the interest-only payment period and the maturity date. In addition, we concurrently increased the outstanding principal by \$10.0 million.

As of August 2018, outstanding borrowings under the 2018 Loan Agreement consisted of a Term A loan, in the amount of \$20.0 million.

In February 2019, we drew the remaining available balance under the 2018 Loan Agreement, or Term B loan, in the amount of \$10.0 million. The Term A loan and Term B loan (together "Term Loans") bear interest at a floating per annum rate equal to (1) 6.94% plus (2) the greater of (a) the 30 day U.S. Dollar LIBOR rate reported in The Wall Street Journal on the last business day of the month that immediately precedes the month in which the interest will accrue and (b) 2.00%. As of December 31, 2020, the interest rate applicable to our Term Loans borrowing was 8.9%.

We will be required to make a final payment of 7.5% of the principal balance outstanding, payable on the earlier of (1) the maturity date, (2) acceleration of any Term Loan or (3) the prepayment of the Term Loans.

In June 2020, we entered into a fourth amendment to the 2018 Loan Agreement with Oxford to extend the interest-only payment period and maturity date. All outstanding Term Loans will now mature on June 1, 2024 and will have interest-only payments through December 31, 2021, followed by 30 equal monthly payments of principal and unpaid accrued interest. In addition, in conjunction with the entry into the fourth amendment, we paid Oxford a facility fee of \$0.3 million. Our obligations under the 2018 Loan Agreement are secured by a first priority security interest in substantially all of our current and future assets, other than our intellectual property. In addition, we have also agreed not to encumber our intellectual property assets, except as permitted by the 2018 Loan Agreement. While any amounts are outstanding under the 2018 Loan Agreement, we are subject to a number of affirmative and restrictive covenants, including covenants regarding dispositions of property, business combinations or acquisitions, among other customary covenants. We are also restricted from paying dividends or making other distributions or annual payments on our capital stock in excess of \$250,000, subject to limited exceptions. In July 2017, the U.K.'s Financial Conduct Authority, which regulates the London Interbank Offered Rate, or LIBOR, announced that it intends to phase out LIBOR by the end of 2021. On November 30, 2020, ICE Benchmark Administration, with the support of the United States Federal Reserve and the FCA, announced plans to consult on ceasing publication of USD LIBOR on December 31, 2021 for only the one week and two month USD LIBOR tenors, and on June 30, 2023 for all other USD LIBOR tenors. Various central bank committees and working groups continue to discuss replacement of benchmark rates, the process for amending existing LIBOR-based contracts, and the potential economic impacts of different alternatives. The Alternative Reference Rates Committee has identified the Secured Overnight Financing Rate, or SOFR, as its preferred alternative rate for USD LIBOR. SOFR is a measure of the cost of borrowing cash overnight, collateralized by U.S. Treasury securities, and is based on directly observable U.S. Treasury-backed repurchase transactions.

We are evaluating the potential impact of the replacement of the LIBOR benchmark interest rate including risk management, internal operational readiness and monitoring the Financial Accounting Standards Board standard-setting process to address financial reporting issues that might arise in connection with the transition from LIBOR to a new benchmark rate.

Cash Flows

The following table sets forth the primary sources and uses of cash and cash equivalents for the years ended December 31, 2020 and 2019 (in thousands):

	Year Ended December 31,	
	2020	2019
Cash used in operating activities	\$ (113,328)	\$ (64,518)
Cash used in investing activities	(204,431)	(42,464)
Cash provided by financing activities	313,941	164,371
Net increase (decrease) in cash and cash equivalents	<u>\$ (3,818)</u>	<u>\$ 57,389</u>

During the year ended December 31, 2020, operating activities used \$113.3 million of cash, primarily resulting from our net loss of \$129.8 million, offset by non-cash expenses of \$10.8 million, and net cash provided by changes in our operating assets and liabilities of \$5.6 million. Non-cash charges consisted primarily of \$7.2 million in stock-based compensation, \$2.6 million in depreciation expense, \$0.8 million of accretion of discount on issued term debt, and \$0.4 million from change in fair value of the preferred stock warrant liability offset by \$0.2 million in accretion of investment discount, net. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2020 consisted primarily of a \$10.3 million increase in accrued liabilities and a \$3.1 million decrease in operating lease right-of-use assets offset by a \$4.2 million decrease in accounts payable, a \$3.3 million increase in prepaid expenses and other current assets, a \$0.2 million increase in other long-term assets, and a \$0.1 million decrease in operating lease liabilities.

During the year ended 2019, operating activities used \$64.5 million of cash, primarily resulting from our net loss of \$86.5 million, offset in part by non-cash charges of \$12.6 million. Non-cash charges consisted primarily of a \$6.7 million increase in contingent consideration, \$3.1 million of stock-based compensation and \$1.2 million of depreciation and amortization. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2019 consisted primarily of a \$8.7 million increase in accounts payable and accrued liabilities.

Cash Used in Investing Activities

During the year ended December 31, 2020, net cash used in investing activities was \$204.4 million, consisting of purchases of short-term investments of \$295.0 million and purchases of property and equipment of \$16.9 million, offset by proceeds from maturities of short-term investments of \$107.5 million. The purchase of short-term investments reflects the use of a higher cash balance from the proceeds of the IPO and Series D redeemable convertible preferred stock financing.

During the year ended December 31, 2019, net cash used in investing activities was \$42.5 million, primarily due to the purchase of \$71.3 million of short-term investments and \$5.2 million of property and equipment purchases, offset in part by \$34.0 million of proceeds from the maturities of available-for-sale short-term investments. The purchase of short-term investments reflects the use of a higher cash balance from the proceeds of the Series C redeemable convertible preferred stock financing.

The timing of purchase and sales of our short-term investments is driven by available cash balance and maturity of existing investments. The purchase of property and equipment for all periods related to equipment purchases as we expanded our research and development and manufacturing activities, in addition to corporate office space.

Cash Provided by Financing Activities

During the year ended December 31, 2020, net cash provided by financings activities was \$313.9 million, consisting primarily of \$205.7 million in net proceeds from the IPO, \$104.1 million in net proceeds from the sale of preferred stock and \$4.2 million in grant payments from CIRM.

During the year ended December 31, 2019, net cash provided by financings activities was \$164.4 million, consisting primarily of \$149.7 million in net proceeds from the sale of preferred stock, \$9.9 million of net proceeds from borrowings under our 2018 Loan Agreement and \$4.6 million in grant payments from CIRM.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations and commitments as of December 31, 2020 (in thousands):

	Payments Due by Period				
	Total	Less than 1 Year	1 to 3 Years	4 to 5 Years	More than 5 Years
Operating lease commitments	\$ 42,469	\$ 3,938	\$ 13,393	\$ 9,612	\$ 15,526
Debt obligations	38,481	2,719	35,762	-	-
Total	<u>\$ 80,950</u>	<u>\$ 6,657</u>	<u>\$ 49,155</u>	<u>\$ 9,612</u>	<u>\$ 15,526</u>

We enter into contracts in the normal course of business with contract research organizations, CMOs and other third parties for preclinical research studies, clinical trials and testing and manufacturing services. These contracts do not contain minimum purchase commitments and are cancelable by us upon prior written notice. Payments due upon cancellation consist of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to one year after the date of cancellation. These payments are not included in the table above as the amount and timing of such payments are not known.

We have also entered into several license agreements under which we are obligated to make aggregate milestone payments upon the achievement of specified preclinical, clinical and regulatory milestones as well as royalty payments. We have not included future payments under this agreement in the table above since the payment obligations under this agreement are contingent upon future events, such as our achievement of specified milestones or generating product sales. As of December 31, 2020, we were unable to estimate the timing or likelihood of achieving these milestones or generating future product sales. See the subsection titled “—License Agreements” above.

Critical Accounting Policies and Significant Judgments and Estimates

Management’s discussion and analysis of our financial condition and results of operations are based upon our financial statements, which are prepared in accordance with accounting principles that are generally accepted in the United States. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets and liabilities, related disclosure of contingent liabilities at the date of the financial statements, and the reported amounts of expenses and other income during the reporting period. We continually evaluate our estimates and judgments, the most critical of which are those related to preclinical and clinical study accruals and stock-based compensation costs. We base our estimates and judgments on historical experience and other factors that we believe to be reasonable under the circumstances. Materially different results can occur as circumstances change and additional information becomes known.

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements, we believe the following accounting policies and estimates to be most critical to the preparation of our consolidated financial statements.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services, however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of the estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include fees paid to vendors in connection with preclinical development activities, CMOs in connection with the process development and scale-up activities and the production of clinical trial materials and contract research organizations in connection with clinical trials.

We base the expense recorded related to contract research and manufacturing on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CMOs and contract research organizations that supply materials and conduct services. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

Stock-Based Compensation

We measure stock-based awards granted to employees, non-employees and directors based on their fair value on the date of the grant using the Black-Scholes option-pricing model for options. Compensation expense for those awards is recognized over the requisite service period, which is generally the vesting period of the respective award. We use the straight-line method to record the expense of awards with service-based vesting conditions. Forfeitures are recognized as they occur.

The Black-Scholes option-pricing model requires the use of subjective assumptions to determine the fair value of stock-based awards. These assumptions include:

- *Fair value of common stock*—Determined based on the quoted market price of our common stock
- *Expected term*—The expected term represents the period that stock-based awards are expected to be outstanding. The expected term for option grants is determined using the simplified method. The simplified method deems the expected term to be the midpoint between the vesting date and the contractual life of the stock-based awards.
- *Expected volatility*—Since we were a privately held company until July 2020 and do not have significant trading history for our common stock, the expected volatility is estimated based on the average volatility for comparable publicly traded biotechnology companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on their similar size, stage in the life cycle or area of specialty. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available.
- *Risk-free interest rate*—The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of option.
- *Expected dividend*—We have never paid dividends on our common stock and have no plans to pay dividends on our common stock. Therefore, we used an expected dividend yield of zero.

As of December 31, 2020, the unrecognized stock-based compensation expense related to employee stock options was \$23.0 million and is expected to be recognized as expense over a weighted-average period of approximately 3.0 years. The intrinsic value of all outstanding stock options as of December 31, 2020 was approximately \$10.0 million, of which approximately \$8.2 million related to vested options and approximately \$1.8 million related to unvested options.

Valuation of Warrants to Purchase Preferred Stock

Prior to the IPO, we classified warrants to purchase shares of our Series A-1 preferred stock and Series B preferred stock as a liability on our consolidated balance sheets as these warrants are free-standing financial instruments that may have required us to transfer assets upon exercise. The warrants were initially recorded at fair value on the date of grant, and they were subsequently remeasured to fair value at each balance sheet date. Changes in fair value of the warrants were recognized as a component of other income (expense) in our consolidated statements of operations and comprehensive loss.

We utilized the Black-Scholes option-pricing model, which incorporates assumptions and estimates to value the preferred stock warrants. We assessed these assumptions and estimates on a quarterly basis as additional information impacting the assumptions is obtained. Similar to the fair value measurement of our common stock prior to IPO, estimates and assumptions impacting the fair value measurement of our preferred stock warrants included the fair value per share of the underlying Series A-1 preferred stock and Series B preferred stock, the remaining contractual term of the warrants, the expected volatility of the price of the underlying preferred stock, the risk-free interest rate and the expected dividend yield. The most significant assumption in the Black-Scholes option-pricing model impacting the fair value of the preferred stock warrants was the fair value of our preferred stock as of each remeasurement date. We determined the fair value per share of the underlying preferred stock by taking into consideration our most recent sales of our preferred stock as well as additional factors that we deemed relevant (including the various factors analyzed to determine the fair value of our common stock described in the subsection titled “—Determination of Fair Value of Common Stock” above).

Upon the closing of the IPO, the preferred stock warrants became exercisable for common stock instead of preferred stock and the fair value of the warrant liability at the date of the IPO was reclassified to additional paid-in-capital. See Note 5 to our annual consolidated financial statements.

Off-Balance Sheet Arrangements

Since our inception, we have not engaged in any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

JOBS Act

The JOBS Act permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. We have elected to use this the extended transition period under the JOBS Act until the earlier of the date we (1) are no longer an emerging growth company or (2) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, our consolidated financial statements may not be comparable to companies that comply with new or revised accounting pronouncements as of public company effective dates.

We will remain an emerging growth company until the earliest to occur of: (1) the last day of the fiscal year in which we have more than \$1.07 billion in annual revenue; (2) the date we qualify as a “large accelerated filer,” with at least \$700.0 million of equity securities held by non-affiliates; (3) the date on which we have issued more than \$1.0 billion in non-convertible debt securities during the prior three-year period; and (4) December 31, 2025.

Recent Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position, results of operations or cash flows is disclosed in Note 2 to our consolidated financial statements.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

As of December 31, 2020, we had cash, cash equivalents and short-term investments of \$309.2 million. Cash consists of deposits with financial institutions. Interest income is sensitive to changes in the general level of interest rates. However, due to the nature of these investments, a hypothetical 10% change in interest rates during any of the periods presented would not have had a material impact on our consolidated financial statements.

As of December 31, 2020, we had \$30.0 million of borrowings outstanding under the 2018 Loan Agreement bearing interest at a variable rate equal to 30-day LIBOR plus 6.94%, subject to a floor of 8.94%. LIBOR is currently scheduled to be phased out on June 30, 2023. Before LIBOR is phased out, we may need to renegotiate the Term Loans to replace LIBOR with a new standard, which has yet to be established. The consequences of these developments cannot be entirely predicted, but could result in higher interest rates on the principal amount of the Term Loans. A hypothetical 10% change in interest rates during any of the periods presented would not have had a material impact on our consolidated financial statements.

Foreign Currency Exchange Risk

To date, foreign currency transaction gains and losses have not been material to our consolidated financial statements, and we have not had a formal hedging program with respect to foreign currency. Our expenses are generally denominated in U.S. dollars. However, we have contracted with a limited number of foreign vendors located in Europe and Canada and may contract with foreign vendors in the future. Our operations may be subject to fluctuations in foreign currency exchange rates in the future. A hypothetical 10% change in exchange rates during any of the periods presented would not have had a material impact on our consolidated financial statements.

Effects of Inflation

Inflation generally affects us by increasing our cost of labor. We do not believe that inflation had a material effect on our consolidated financial statements.

Item 8. Financial Statements and Supplementary Data.

The financial statements and supplemental data required by this item are set forth at the pages indicated in Part IV, Item 15 of this Annual Report on Form 10-K.

Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

We are responsible for maintaining disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act. Disclosure controls and procedures are designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported, within the time periods specified in the SEC's rules and forms and that such information is accumulated and communicated to our management, including our Principal Executive Officer and Principal Financial Officer, as appropriate to allow timely decisions regarding required disclosure.

Based on our management's evaluation, the Principal Executive Officer and Principal Financial Officer have concluded that our disclosure controls and procedures were effective as of December 31, 2020.

Internal Control Over Financial Reporting

This Annual Report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of the company's registered public accounting firm due to a transition period established by rules of the Securities and Exchange Commission for newly public companies.

Remediation of Previously Identified Material Weaknesses

We previously identified material weaknesses in our internal control over financial reporting.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim consolidated financial statements will not be prevented or detected on a timely basis.

These material weaknesses related to a lack of a sufficient complement of accounting resources, which led to our inability to maintain segregation of duties between the creation and posting of journal entries and review of account reconciliations. These material weaknesses did not result in a misstatement to our consolidated financial statements.

In order to remediate these material weaknesses, actions were taken to strengthen our controls, including the hiring of additional internal accounting resources and the engagement of additional third-party resources. During the quarter ending September 30, 2020, we updated access controls to remove the ability to both create and post journal entries. We also evaluated the design of all processes to ensure with the expansion of the accounting team, appropriate segregation of duties exist for all key controls and appropriate review of account reconciliations were performed. Based on the actions taken, as well as the testing and evaluation of the design and operating effectiveness of these controls, we concluded that the material weaknesses previously identified were remediated as of December 31, 2020.

Changes in Internal Control Over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended December 31, 2020 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

None.

Item 10. Directors, Executive Officers and Corporate Governance.

The information required by this item (other than as set forth below) is incorporated herein by reference to our Proxy Statement with respect to our 2021 Annual Meeting of Stockholders to be filed with the SEC within 120 days of the end of the fiscal year covered by this Annual Report on Form 10-K.

We have adopted a written code of business conduct and ethics that applies to all our directors, officers and employees, including our principal executive officer, principal financial officer and principal accounting officer. A current copy of the code of business conduct and ethics is available on the Corporate Governance section of our website at www.poseida.com. We intend to disclose on our website any amendments to, or waivers from, our code of business conduct and ethics that are required to be disclosed pursuant to SEC rules.

Item 11. Executive Compensation.

The information required by this item is incorporated herein by reference to our Proxy Statement with respect to our 2021 Annual Meeting of Stockholders to be filed with the SEC within 120 days of the end of the fiscal year covered by this Annual Report on Form 10-K.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required by this item is incorporated herein by reference to our Proxy Statement with respect to our 2021 Annual Meeting of Stockholders to be filed with the SEC within 120 days of the end of the fiscal year covered by this Annual Report on Form 10-K.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this item is incorporated herein by reference to our Proxy Statement with respect to our 2021 Annual Meeting of Stockholders to be filed with the SEC within 120 days of the end of the fiscal year covered by this Annual Report on Form 10-K.

Item 14. Principal Accounting Fees and Services.

The information required by this item is incorporated herein by reference to our Proxy Statement with respect to our 2021 Annual Meeting of Stockholders to be filed with the SEC within 120 days of the end of the fiscal year covered by this Annual Report on Form 10-K.

Item 15. Exhibits, Financial Statement Schedules.

We have filed the following financial statements and financial statement schedules as part of this Annual Report:

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm	146
Consolidated Balance Sheets as of December 31, 2020 and 2019	147
Consolidated Statements of Operations and Comprehensive Loss for the Years ended December 31, 2020 and 2019	148
Consolidated Statements of Changes in Convertible Preferred Stock and Stockholders' Equity (Deficit) for the Years ended December 31, 2020 and 2019	149
Consolidated Statements of Cash Flows for the Years ended December 31, 2020 and 2019	150
Notes to Consolidated Financial Statements	151

Exhibits

The exhibits listed in the accompanying index to exhibits are filed or incorporated by reference as part of this Annual Report on Form 10-K.

To the Board of Directors and Stockholders of Poseida Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Poseida Therapeutics, Inc. and its subsidiaries (the “Company”) as of December 31, 2020 and December 31, 2019, and the related consolidated statements of operations and comprehensive loss, of changes in convertible preferred stock and stockholders’ equity (deficit) and of cash flows for each of the two years in the period ended December 31, 2020, including the related notes (collectively referred to as the “consolidated financial statements”). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2020 and 2019, and the results of its operations and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America.

Change in Accounting Principle

As discussed in Note 2 to the consolidated financial statements, the Company changed the manner in which it accounts for leases in 2020.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits of these consolidated financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP

San Diego, California
March 11, 2021

We have served as the Company's auditor since 2015.

Poseida Therapeutics, Inc.
CONSOLIDATED BALANCE SHEETS
(In thousands, except share amounts)

	December 31,	
	2020	2019
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 83,966	\$ 87,784
Short-term investments	225,186	37,534
Prepaid expenses and other current assets	4,844	1,861
Total current assets	313,996	127,179
Property and equipment, net	23,336	10,858
Operating lease right-of-use assets	24,986	—
Intangible assets, net	1,320	1,320
Goodwill	4,228	4,228
Other long-term assets	3,618	3,411
Total assets	\$ 371,484	\$ 146,996
LIABILITIES, CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current liabilities:		
Accounts payable	\$ 763	\$ 4,929
Accrued and other liabilities	24,454	13,926
Operating lease liabilities, current	4,808	—
Term debt - short-term	—	3,000
Total current liabilities	30,025	21,855
Term debt - long-term	29,133	26,140
Deferred CIRM grant liability	23,755	19,592
Warrant liability	—	1,271
Deferred tax liability	55	55
Operating lease liabilities, non-current	25,374	—
Other long-term liabilities	1,174	5,421
Total liabilities	109,516	74,334
<i>Commitments and contingencies (Note 10)</i>		
Convertible preferred stock (Series A, A-1, B, C and D), \$0.0001 par value: 0 and 33,085,827 shares authorized at December 31, 2020 and December 31, 2019, respectively; 0 and 32,934,785 shares issued and outstanding at December 31, 2020 and December 31, 2019, respectively	—	222,173
Stockholders' equity (deficit):		
Common stock, \$0.0001 par value: 250,000,000 and 57,013,463 shares authorized at December 31, 2020 and December 31, 2019, respectively; 61,860,897 and 13,196,419 shares issued and outstanding as of December 31, 2020 and December 31, 2019, respectively	6	2
Additional paid-in capital	543,842	2,689
Accumulated other comprehensive income	5	19
Accumulated deficit	(281,885)	(152,221)
Total stockholders' equity (deficit)	261,968	(149,511)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	\$ 371,484	\$ 146,996

The accompanying notes are an integral part of these financial statements.

Poseida Therapeutics, Inc.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(In thousands, except share and per share amounts)

	Year Ended December 31,	
	2020	2019
Operating expenses:		
Research and development	\$ 103,520	\$ 60,393
General and administrative	23,029	18,457
Increase in contingent consideration (inclusive of related party amounts of \$0 and \$2,739, respectively)	—	6,683
Total operating expenses	<u>126,549</u>	<u>85,533</u>
Loss from operations	(126,549)	(85,533)
Other income (expense):		
Interest expense	(3,506)	(3,553)
Other income, net	280	2,559
Net loss before income tax	(129,775)	(86,527)
Income tax benefit	—	—
Net loss	<u>\$ (129,775)</u>	<u>\$ (86,527)</u>
Other comprehensive income (expense):		
Other comprehensive income (loss) (net of tax expense of \$0 for each of the periods ending December 31, 2020 and 2019)	\$ (14)	\$ 19
Total other comprehensive income (loss)	<u>\$ (14)</u>	<u>\$ 19</u>
Comprehensive loss	<u>\$ (129,789)</u>	<u>\$ (86,508)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (3.61)</u>	<u>\$ (6.86)</u>
Weighted-average shares of common stock, basic and diluted	<u>35,996,901</u>	<u>12,618,413</u>

The accompanying notes are an integral part of these financial statements.

Poseida Therapeutics, Inc.
CONSOLIDATED STATEMENTS OF CHANGES IN CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)
(In thousands, except share amounts)

	Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
Balance at January 1, 2019	18,200,011	\$ 72,460	12,275,579	\$ 2	\$ (11,026)	\$ —	\$ (65,694)	\$ (76,718)
Net loss	—	—	—	—	—	—	(86,527)	(86,527)
Issuance of common stock under employee stock compensation plans	—	—	54,715	—	69	—	—	69
Issuance of common stock for acquisition of Vindico	—	—	866,125	—	10,596	—	—	10,596
Issuance of Series C preferred stock for cash, net of issuance costs \$287	14,734,774	149,713	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	3,050	—	—	3,050
Unrealized gain on marketable securities	—	—	—	—	—	19	—	19
Balance at December 31, 2019	32,934,785	\$ 222,173	13,196,419	\$ 2	\$ 2,689	\$ 19	\$ (152,221)	\$ (149,511)
Net loss	—	—	—	—	—	—	(129,775)	(129,775)
Transition adjustment from adoption of ASC 842 (Note 2)	—	—	—	—	—	—	111	111
Issuance of common stock under employee stock compensation plans	—	—	219,370	—	224	—	—	224
Issuance of Series D preferred stock for cash, net of issuance costs \$5,359	10,018,300	104,140	—	—	—	—	—	—
Issuance of common stock for conversion of preferred stock in closing of IPO	(42,953,085)	(326,313)	34,445,108	3	326,309	—	—	326,312
Issuance of common stock from initial public offering, net of issuance costs of \$18,018	—	—	14,000,000	1	205,742	—	—	205,743
Stock-based compensation expense	—	—	—	—	7,220	—	—	7,220
Reclassification of Series A-1 and Series B warrants	—	—	—	—	1,658	—	—	1,658
Unrealized gain on marketable securities	—	—	—	—	—	(14)	—	(14)
Balance at December 31, 2020	—	\$ —	61,860,897	\$ 6	\$ 543,842	\$ 5	\$ (281,885)	\$ 261,968

The accompanying notes are an integral part of these financial statements.

Poseida Therapeutics, Inc.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	Year Ended December 31,	
	2020	2019
OPERATING ACTIVITIES		
Net loss	\$ (129,775)	\$ (86,527)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization expense	2,586	1,193
Loss on disposal of assets	—	448
Stock-based compensation	7,220	3,050
Change in fair value of contingent liabilities	—	6,679
Change in fair value of warrant liability	387	(519)
Accretion of discount on issued term debt	805	853
Accretion of investment discount, net	(174)	(128)
Imputed rent expense	—	111
Write off of deferred financing costs	—	855
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(3,296)	726
Operating lease right-of-use assets	3,108	—
Other long-term assets	(207)	(1,676)
Accounts payable	(4,171)	1,530
Accrued liabilities	10,320	7,132
Operating lease liabilities	(131)	—
Other long-term liabilities	—	1,755
Net cash used in operating activities	<u>(113,328)</u>	<u>(64,518)</u>
INVESTING ACTIVITIES		
Purchases of property and equipment	(16,908)	(5,156)
Purchases of short-term investments	(295,023)	(71,308)
Proceeds from maturities of short-term investments	107,500	34,000
Net cash used in investing activities	<u>(204,431)</u>	<u>(42,464)</u>
FINANCING ACTIVITIES		
Net proceeds from stock option exercises	223	69
Issuance of Series C financing, net of issuance costs	—	149,713
Issuance of Series D financing, net of issuance costs	104,141	—
Proceeds from initial public offering, net of issuance costs	205,743	—
Net proceeds from CIRM	4,163	4,642
Proceeds from term debt	—	10,000
Payment of debt issuance costs	(329)	(53)
Net cash provided by financing activities	<u>313,941</u>	<u>164,371</u>
Net increase (decrease) in cash and cash equivalents	<u>(3,818)</u>	<u>57,389</u>
Cash and cash equivalents at beginning of period	87,784	30,395
Cash and cash equivalents at end of period	<u>\$ 83,966</u>	<u>\$ 87,784</u>
Non-cash investing and financing activities:		
Issuance of common stock for acquisition	<u>\$ —</u>	<u>\$ 10,596</u>
Purchases of property and equipment included in accounts payable and accrued liabilities	<u>\$ 211</u>	<u>\$ 1,887</u>
Tenant improvement receivable from landlord	<u>\$ 137</u>	<u>\$ 421</u>
Construction financing liability	<u>\$ —</u>	<u>\$ 2,166</u>
Right-of-use assets and lease liabilities	<u>\$ 5,346</u>	<u>\$ —</u>
Conversion of redeemable convertible preferred stock to common stock upon closing of initial public offering	<u>\$ 326,313</u>	<u>\$ —</u>
Supplemental disclosure of cash flow information:		
Interest paid	<u>\$ 3,027</u>	<u>\$ 2,631</u>

The accompanying notes are an integral part of these financial statements.

Poseida Therapeutics, Inc.
Notes to Consolidated Financial Statements

Note 1. Nature of Business and Basis of Presentation

Nature of Operations

Poseida Therapeutics, Inc. (the “Company” or “Poseida”) is a clinical-stage biopharmaceutical company dedicated to utilizing its proprietary gene engineering platform technologies to create next generation cell and gene therapeutics with the capacity to cure.

The Company is subject to risks and uncertainties common to development-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company’s therapeutic development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Reverse Stock Split

These consolidated financial statements reflect a 1-for-1.247 reverse stock split of the Company’s common stock, which became effective on July 2, 2020. All share and per share data for all periods presented in the accompanying consolidated financial statements and notes thereto have been adjusted retrospectively, where applicable, to reflect the reverse stock split.

Initial Public Offering

On July 14, 2020, the Company completed its initial public offering (“IPO”), in which the Company issued and sold 14,000,000 shares of its common stock, at a public offering price of \$16.00 per share, for aggregate gross proceeds of \$224.0 million. The Company received approximately \$205.7 million in net proceeds after deducting underwriting discounts and offering expenses payable by the Company. At the closing of the IPO, 42,953,085 shares of outstanding Convertible Preferred Stock were automatically converted into 34,445,108 shares of common stock, and outstanding warrants to purchase an aggregate of 151,042 shares of Convertible Preferred Stock became exercisable for 121,122 shares of common stock and were reclassified into permanent equity. Following the IPO there were no shares of preferred stock outstanding. In connection with the completion of the Company’s IPO in July 2020, the Company amended its Certificate of Incorporation to authorize 250,000,000 shares of common stock, par value \$0.0001 per share, and 10,000,000 shares of undesignated preferred stock that may be issued from time to time by the Company’s board of directors in one or more series, par value of \$0.0001 per share.

Liquidity and Capital Resources

The Company has experienced net losses and negative cash flows from operations since its inception and has relied on its ability to fund its operations primarily through equity financings. The Company has incurred net losses for the years ended December 31, 2020 and 2019 of \$129.8 million and \$86.5 million, respectively, and negative cash flows from operations for these same periods of \$113.3 million and \$64.5 million, respectively. The Company expects to continue to incur net losses and negative cash flows from operations for at least the next several years. As of December 31, 2020, the Company had an accumulated deficit of \$281.9 million.

The Company expects that its cash, cash equivalents and marketable securities as of December 31, 2020 of \$309.2 million will be sufficient to fund its operations for at least the next twelve months from the date of issuance of these financial statements. In the long term the Company will need additional financing to support its continuing operations and pursue its growth strategy. Until such time as the Company can generate significant revenue from product sales, if ever, it expects to finance its operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. The Company may be unable to raise additional funds

or enter into such other agreements when needed on favorable terms or at all. The inability to raise capital as and when needed would have a negative impact on the Company's financial condition and its ability to pursue its business strategy. The Company will need to generate significant revenue to achieve profitability, and it may never do so.

Basis of Preparation and Consolidation

The consolidated financial statements reflect the Company's financial position, results of operations and cash flows, in conformity with generally accepted accounting principles in the United States ("GAAP") and include the accounts of Poseida Therapeutics, Inc. and its wholly-owned subsidiaries. All intercompany transactions and balances have been eliminated.

Note 2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires the Company to make estimates and assumptions that affect the amounts reported in the consolidated financial statements and accompanying notes. On an ongoing basis, the Company evaluates its estimates, which include, but are not limited to, estimates related to accrued expenses, contingent consideration, warrant liability, stock-based compensation expense, deferred tax valuation allowances and, prior to the Company's IPO, the fair value of common stock. The Company bases its estimates on historical experience and other market-specific or relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions.

Prior to the IPO, the Company utilized significant estimates and assumptions in determining the fair value of its common stock. The Company has utilized various valuation methodologies in accordance with the framework of the 2004 American Institute of Certified Public Accountants Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*, to estimate the fair value of its common stock. Each valuation methodology includes estimates and assumptions that require the Company's judgment. These estimates and assumptions include a number of objective and subjective factors, including the prices at which the Company sold shares of preferred stock and the superior rights, preferences and privileges of the preferred stock relative to the common stock; the Company's stage of development and material risks related to its business; the progress of the Company's research and development programs, including the status and results of preclinical studies for its product candidates and progress of its development of manufacturing processes; external market conditions affecting the biopharmaceutical industry and trends within the biopharmaceutical industry; the Company's results of operations and financial position, including its levels of available capital resources, outstanding debt and its historical and forecasted performance and operating results; the lack of an active public market for the Company's common stock and preferred stock; the likelihood of achieving a liquidity event, such as an IPO or sale of the Company in light of prevailing market conditions; the hiring of key personnel; and the analysis of IPOs and the market performance of publicly traded companies in the biopharmaceutical industry, as well as recently completed mergers and acquisitions of peer companies. Significant changes to the key assumptions used in the valuations could result in different fair values of common stock at each valuation date.

Segment Information

The Company's sole operations consist of developing therapeutics for patients with high unmet medical need. Accordingly, the Company has determined that it operates in one operating segment. Operating segments are defined as components of an enterprise about which separate financial information is evaluated regularly by the Company's chief operating decision maker, who is its Chief Executive Officer, in deciding how to allocate resources and assess performance. The Company's chief operating decision maker allocates resources and assesses performance based upon discrete financial information at the consolidated level. All of the Company's tangible assets are held in the United States.

Fair Value Measurements

Certain financial instruments are required to be recorded at fair value. Other financial instruments, like cash are recorded at cost, which approximates fair value. Cash equivalents and short-term investments are comprised of available-for-sale securities, which are carried at fair value. Additionally, carrying amounts of accounts payable and accrued liabilities approximate fair value because of the short maturity of those instruments. The carrying value of the Company's term debt approximates its fair value due to its variable interest rate, which approximates a market interest rate.

Concentration of Business Risk

The Company relies, and expects to continue to rely, on a small number of vendors to manufacture supplies and materials for its development programs. These programs could be adversely affected by a significant interruption in these manufacturing services.

Leases

The Company accounts for leases in accordance with Accounting Standards Codification Topic 842, *Leases*, ("ASC 842"). The Company determines if an arrangement is a lease at contract inception. A lease exists when a contract conveys the right to control the use of identified property, plant, or equipment for a period of time in exchange for consideration. The definition of a lease embodies two conditions: (1) there is an identified asset in the contract that is land or a depreciable asset (i.e., property, plant, and equipment), and (2) the Company has the right to control the use of the identified asset.

Operating leases where the Company is the lessee are included in lease receivables, operating lease right-of-use ("ROU") assets, operating lease liabilities, current and operating lease liabilities, non-current on its consolidated balance sheets. The lease liabilities are initially and subsequently measured at the present value of the unpaid lease payments at the lease commencement date.

Lease receivables, included within prepaid and other currents assets within the consolidated balance sheets, are comprised of the expected tenant improvement reimbursement from the landlord and the rent abatement period to be recognized over the following twelve months.

ASC 842 requires a lessee to discount its unpaid lease payments using the interest rate implicit in the lease or, if that rate cannot be readily determined, its incremental borrowing rate. The rates implicit in the Company's leases are not known, therefore, the incremental borrowing rate is used based on the information available at commencement date in determining the present value of lease payments. The Company's incremental borrowing rate for a lease is the rate of interest it would have to pay on a collateralized basis to borrow an amount equal to the lease payments under similar terms.

The lease term for all of the Company's leases includes the noncancelable period of the lease. Where the Company's lease term is impacted by options to extend or terminate the lease, when it is reasonably certain that it will exercise such option, then the lease payments are included in the measurement of the lease asset or liability.

The Company has elected not to recognize ROU assets and lease liabilities for all short-term leases that have a lease term of 12 months or less. The Company recognizes the lease payments associated with its short-term leases as an expense on a straight-line basis over the lease term. There are no variable lease payments associated with these leases. Additionally, the Company has elected to account for the lease and non-lease components together as a single lease component for its real estate asset class.

Risk and Uncertainties

In March 2020, the World Health Organization made the assessment that a novel strain of coronavirus, SARS-CoV-2, a novel strain of coronavirus, commonly referred to as COVID-19 had become a global pandemic. The impact of this pandemic has been and will likely continue to be extensive in many aspects of society, which has

resulted in and will likely continue to result in significant disruptions to the global economy, as well as businesses and capital markets around the world.

Impacts to the Company's business, some of which the Company has already experienced, include, but are not limited to, temporary closures of its facilities or those of its vendors, disruptions or restrictions on its employees' ability to travel, disruptions to or delays in ongoing laboratory experiments, preclinical studies, clinical trials, third-party manufacturing supply and other operations, the diversion of healthcare resources away from the conduct of clinical trials to focus on pandemic concerns, interruptions or delays in the operations of the U.S. Food and Drug Administration or other regulatory authorities, and the Company's ability to raise capital and conduct business development activities.

Deferred Offering Costs

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After consummation of an equity financing, these costs are recorded as a reduction of additional paid-in capital generated as a result of the offering. Should the in-process equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the consolidated statements of operations and comprehensive loss. During the year ended December 31, 2019, the Company expensed \$1.8 million of costs, within general and administrative expenses, previously capitalized and associated with the Company's abandoned efforts to complete an IPO in early 2019. Deferred offering costs of \$18.3 million, incurred in connection with the Company's July 2020 IPO, were net against the gross proceeds on the statement of stockholders' equity (deficit) as of December 31, 2020.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash, cash equivalents and short-term investments. The Company maintains its cash and cash equivalent balances with high-quality financial institutions and, consequently, the Company believes that such funds are subject to minimal credit risk. Deposits held at these institutions may exceed the amount of insurance provided on such deposits.

Cash and Cash Equivalents

The Company considers all highly liquid investments purchased with original final maturities of 90 days or less from the date of purchase to be cash equivalents. Cash and cash equivalents consist of deposits with financial institutions and marketable securities. Cash equivalents are reported at fair value. The Company utilizes a credit card that requires a cash collateral account to secure its outstanding balance. While cash in this account is not legally restricted, the availability of future credit is dependent upon maintenance of a compensating balance sufficient to cover outstanding balances. The balance held in this account as of both December 31, 2020 and 2019 was \$0.2 million. Amounts outstanding on the credit card and recorded as accounts payable were \$0.1 million and zero as of December 31, 2020 and 2019, respectively.

Short-Term Investments

Investments with a remaining maturity when purchased of greater than three months are classified as short-term investments on the balance sheet and consist primarily of U.S. Treasury and government agency obligations. As our entire investment portfolio is considered available for use in current operations, the Company classifies all investment as available-for-sale and as current assets. Debt securities are carried at fair value with the unrealized gains and losses included in other comprehensive income (loss) as a component of stockholders' equity until realized. Any premium or discount arising at purchase is amortized and/or accreted to interest income and/or expense over the life of the instrument. If any adjustment to fair value reflects a decline in the value of the investment that the Company considers to be "other than temporary," the Company reduces the investment to fair value through a charge to the statement of operations and comprehensive loss. No such adjustments were necessary during the periods presented.

Other Receivables, Net

Other receivables is recorded at the invoiced amount and is non-interest bearing. Other receivables, net consists of reimbursements from the Company's landlord for ongoing construction and is included within other current assets.

Goodwill and Other Intangible Assets

Intangible assets were acquired as part of a business combination and have been capitalized at their acquisition date fair value. Indefinite-lived in process research and development ("IPR&D") is not subject to amortization but is tested annually for impairment or more frequently if there are indicators of impairment. The Company tests its indefinite-lived IPR&D annually for impairment during the fourth quarter. In testing indefinite-lived IPR&D for impairment, the Company has the option to first assess qualitative factors to determine whether the existence of events or circumstances would indicate that it is more likely than not that its fair value is less than its carrying amount, or the Company can perform a quantitative impairment analysis to determine the fair value of the indefinite-lived IPR&D without performing a qualitative assessment. Qualitative factors that the Company considers include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If the Company chooses to first assess qualitative factors and it determines that it is more likely than not that the fair value of the indefinite-lived IPR&D is less than its carrying amount, the Company would then determine the fair value of the indefinite-lived IPR&D. Under either approach, if the fair value of the indefinite-lived IPR&D is less than its carrying amount, an impairment charge would be recognized for the difference between the fair value and the carrying amount. There was no impairment of IPR&D for the years ended December 31, 2020 and 2019.

The Company additionally tests its goodwill for impairment annually during the fourth quarter, or whenever events or changes in circumstances indicate an impairment may have occurred. Should an impairment exist, the impairment loss would be measured based on the excess of the carrying amount of the asset or asset group over the estimated asset's fair value. Impairment may result from, among other things, deterioration in the performance of the acquired business, adverse results from developmental work, adverse changes in applicable laws or regulations and a variety of other circumstances. In evaluating the recoverability of the carrying value of goodwill, the Company must make assumptions regarding estimated future cash flows and other factors to determine the fair value of the acquired assets. Changes in strategy or market conditions could significantly impact those judgments in the future and require an adjustment to the recorded balances. There were no impairments of goodwill for the years ended December 31, 2020 and 2019.

Property and Equipment

Property and equipment are stated at cost and depreciated or amortized using the straight-line method, based on their estimated useful lives as follows:

Asset Classification	Estimated Useful Life (years)
Lab equipment	5
Leasehold improvements	Lesser of useful life or lease-term
Computer equipment and software	3
Furniture and fixtures	7

Maintenance and repairs are charged to expense as incurred. When assets are retired or otherwise disposed of, the cost and accumulated depreciation are removed from the Company's consolidated balance sheet and any resulting gain or loss is reflected in the Company's consolidated statement of operations and comprehensive loss.

Property and equipment are reviewed annually for impairment or whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability is measured by comparison of the carrying amount to the future net undiscounted cash flows which the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount

by which the carrying amount of the assets exceeds the projected discounted future net cash flows arising from the asset. There has been no impairment of long-lived assets during the years ended December 31, 2020 and 2019.

Research and Development

Research and development expense consists of labor, material, equipment, and allocated facilities costs of the Company's scientific staff who are working on research and development projects. Research and development costs are charged to operations as incurred.

Upfront payments and milestone payments made for the licensing of technology are expensed as research and development in the period in which they are incurred. Advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses or other long-term assets. The advanced payments are expensed as the related goods are delivered or the services are performed.

Research and Manufacturing Contract Costs and Accruals

The Company has entered into various research and development and manufacturing agreements. These agreements are generally cancelable, and related payments are recorded as the corresponding expenses are incurred. The Company records accruals for estimated costs incurred to date. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the research studies or clinical trials and manufacturing activities, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Stock-Based Compensation

Equity awards to employees are measured and recognized in the consolidated financial statements based on the fair value of the award on the grant date. The Company currently uses the Black-Scholes valuation model to estimate the grant date fair value of their share-based payments. The model requires the Company to make a number of assumptions including expected volatility, risk-free interest rate, expected term and expected dividend. Stock-based compensation expense is recognized straight-line over the term of the option grant. All option grants require continued service to continue vesting. Forfeitures are recognized as they occur.

The Company recognizes the fair value of stock options granted to non-employees as stock-based compensation expense over the period in which the related services are received. Stock-based compensation expense related to stock options granted to non-employees is recognized based on the grant date fair value of awards as the stock options are earned. The Company believes that the estimated fair value of stock options is more readily measurable than the fair value of the services rendered.

Contingent Consideration

In connection with the Company's acquisition of Vindico in October 2016, the Company agreed to pay additional consideration based on the achievement of a certain developmental milestone using the acquired technology. The additional purchase consideration was payable in shares of its common stock. The number of shares of common stock issuable and the associated fair value could vary depending on (1) the price paid by investors in a qualified equity financing prior to the achievement of the milestone and (2) when and if the milestone was reached. Prior to the milestone being met, the Company classified this contingent consideration as a liability on its consolidated balance sheet that was remeasured to fair value at each reporting date, and recognized changes in the

fair value of the contingent consideration liability as a component of operating expenses in its consolidated statements of operation and comprehensive loss. The Company recognized changes in the fair value of the contingent consideration liability until the milestone was met in July 2019. Upon issuance of the common stock related to the milestone in July 2019, the liability was reclassified to stockholder's deficit, within additional paid-in capital.

Comprehensive Loss

Comprehensive loss includes net loss as well as other comprehensive income (loss). Other comprehensive income (loss) consists of unrealized gains and losses on marketable securities.

Net Income (Loss) Per Share

Basic net loss per share is calculated by dividing the net loss by the weighted-average number of shares of common stock outstanding during the period without consideration for common stock equivalents. Diluted net loss per share is the same as basic net loss per common share, since the effects of potentially dilutive securities are anti-dilutive due to the net loss position of all periods presented.

Income Taxes

Deferred tax assets/liabilities are determined based on the difference between the financial statement carrying amounts of assets and liabilities and their respective tax bases, as well as net operating losses and credit carry forwards applied by the enacted tax rates expected to be in effect for the year in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in the consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Emerging Growth Company Status

The Company is an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"). Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. The Company has elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date that it (i) is no longer an emerging growth company or (ii) affirmatively and irrevocably opts out of the extended transition period provided in the JOBS Act. As a result, these consolidated financial statements may not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

Recently Adopted Accounting Pronouncements

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842)*. The FASB subsequently issued ASU 2018-10 *Codification Improvements to Topic 842, Leases*, ASU 2018-11, *Leases (Topic 842): Targeted*

Improvements, and ASU 2019-01, *Leases (Topic 842): Codification Improvements*, to further amend ASU 2016-02. ASU 2016-02, as amended, provides revised guidance related to the accounting and reporting of leases, including a requirement for lessees to recognize most leases on the balance sheet. The recognition, measurement and presentation of expenses and cash flows arising from a lease by a lessee depends on its classification as a finance or operating lease. For public entities, the guidance is effective for fiscal years beginning after December 15, 2018, and for non-public entities, the guidance was effective for fiscal years beginning after December 15, 2020, with early adoption permitted. Companies may adopt retrospectively as of the earliest period presented or retrospectively at the beginning of the period of adoption through a cumulative-effect adjustment, in each case with a number of practical expedients that entities may elect to apply. The Company adopted this standard on January 1, 2020, early adopting ASC 842 using a modified retrospective transition approach as of the effective date, as permitted by the amendments in ASU 2018-11, which provides an alternative modified retrospective transition method. As a result, the Company was not required to adjust its comparative period financial information for effects of the standard or make the new required lease disclosures for periods before the date of adoption. The Company has elected to adopt the package of transition practical expedients and, therefore, it has not reassessed (1) whether existing or expired contracts contain a lease, (2) lease classification for existing or expired leases or (3) the accounting for initial direct costs that were previously capitalized. The Company did not elect the practical expedient to use hindsight for leases existing at the adoption date. See Note 10 for the adoption impact.

In August 2018, the FASB issued ASU 2018-15, *Intangibles—Goodwill and Other—Internal-Use Software (Topic 350): Customer’s Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract*. This standard requires capitalizing implementation costs incurred to develop or obtain internal-use software (and hosting arrangements that include an internal-use software license). The Company adopted this standard on January 1, 2020 using the prospective method. The adoption of this standard did not have a material impact on our consolidated financial statements and disclosures.

In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework – Changes to the Disclosure Requirements for Fair Value Measurement*. The new standard removes certain disclosures, modifies certain disclosures and adds additional disclosures related to fair value measurement. The Company adopted this standard on January 1, 2020 using the prospective method. The adoption of this standard did not have a material impact on our consolidated financial statements and disclosures.

Recently Issued Accounting Pronouncements

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments — Credit Losses*, to improve financial reporting by requiring timely recording of credit losses on loans and other financial instruments held by financial institutions and other organizations. The ASU requires the measurement of all expected credit losses for financial assets held at the reporting date based on historical experience, current conditions and reasonable and supportable forecasts. This guidance will become effective for the Company beginning January 1, 2023, with early adoption permitted. The Company is currently evaluating the potential impact ASU 2016-13 may have on its financial position and results of operations upon adoption.

In December 2019, the FASB issued ASU 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes (“ASU 2019-12”)*, which is intended to simplify the accounting for income taxes. ASU 2019-12 removes certain exceptions to the general principles in Topic 740 and also clarifies and amends existing guidance to improve consistent application. The new standard will be effective for the Company beginning January 1, 2021. The Company is currently evaluating the potential impact ASU 2019-12 may have on its financial position and results of operations upon adoption.

Reclassification

Certain amounts in the consolidated financial statements have been reclassified from their original presentation to conform to current year presentation.

Note 3. Composition of Certain Balance Sheet Components

Prepaid expenses and other current assets

Prepaid expenses and other current assets consist of the following as of (in thousands):

	December 31,	
	2020	2019
Landlord reimbursement	\$ 137	\$ 421
Rent	217	259
Insurance	2,226	204
Contract research services	651	543
Other	1,613	434
Total prepaid expenses and other current assets	<u>\$ 4,844</u>	<u>\$ 1,861</u>

Property and equipment, net

Property and equipment, net consist of the following as of (in thousands):

	December 31,	
	2020	2019
Lab equipment	\$ 11,420	\$ 6,957
Leasehold improvements	13,826	165
Computer equipment and software	1,381	456
Furniture and fixtures	928	560
Construction in progress	376	4,729
Total property and equipment	27,931	12,867
Less: Accumulated depreciation and amortization	(4,595)	(2,009)
Total property and equipment, net	<u>\$ 23,336</u>	<u>\$ 10,858</u>

Depreciation expense associated with property and equipment was \$2.6 million and \$1.2 million for the years ended December 31, 2020 and 2019, respectively.

Goodwill and other intangible assets, net

Goodwill and other intangible assets, net consist of the following as of (in thousands):

	December 31,	
	2020	2019
Goodwill	\$ 4,228	\$ 4,228
Indefinite lived intangible assets:		
IPR&D	\$ 1,320	\$ 1,320
Total intangible assets, net	<u>\$ 1,320</u>	<u>\$ 1,320</u>

There were no impairments of goodwill for the years ended December 31, 2020 and 2019.

Accrued and other liabilities

Accrued and other liabilities consisted of the following as of (in thousands):

	December 31,	
	2020	2019
Contract research services	\$ 15,822	\$ 7,993
Payroll and related expense	6,793	3,283
Lease cancellation fee	—	979
Other	1,839	1,671
Total accrued and other liabilities	\$ 24,454	\$ 13,926

Note 4. Financial Instruments

The following table summarizes the amortized cost and fair value of securities available-for-sale at December 31, 2020 and 2019 (in thousands):

	Amortized Cost/Cost	Unrealized Gains	Unrealized Losses	Fair Value
At December 31, 2020:				
Money market fund	\$ 70,713	\$ —	\$ —	\$ 70,713
U.S. government agency securities and treasuries	225,181	5	—	225,186
Total	\$ 295,894	\$ 5	\$ —	\$ 295,899
At December 31, 2019:				
Money market fund	\$ 63,744	\$ —	\$ —	\$ 63,744
U.S. government agency securities and treasuries	42,503	19	—	42,522
Total	\$ 106,247	\$ 19	\$ —	\$ 106,266

No available-for-sale debt securities held as of December 31, 2020 and 2019 had remaining maturities greater than one year. Unrealized gains and losses on available-for-sale securities are included as a component of comprehensive loss. At December 31, 2020, the Company did not have any securities in material unrealized loss positions. The Company reviews its investments to identify and evaluate investments that have an indication of possible other-than-temporary impairment. Factors considered in determining whether a loss is other-than-temporary include the length of time and extent to which fair value has been less than the cost basis, the financial condition and near-term prospects of the investee, and the Company's intent and ability to hold the investment for a period of time sufficient to allow for any anticipated recovery in market value. The Company does not intend to sell any investments prior to recovery of their amortized cost basis for any investments in an unrealized loss position.

Note 5. Fair Value Measurement

Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants as of the measurement date. Applicable accounting guidance provides an established hierarchy for inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the most observable inputs be used when available. Observable inputs are inputs that market participants would use in valuing the asset or liability and are developed based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the factors that market participants would use in valuing the asset or liability. There are three levels of inputs that may be used to measure fair value:

- Level 1: Unadjusted quoted prices in active markets that are accessible at the measurement date for identical, unrestricted assets or liabilities

- Level 2: Significant other observable inputs other than Level 1 prices such as quoted prices in markets that are not active, or inputs that are observable, either directly or indirectly, for substantially the full term of the asset or liability
- Level 3: Prices or valuation techniques that require inputs that are both significant to the fair value measurement and unobservable (i.e., supported by little or no market activity)

The Company classifies its money market funds and U.S. treasury securities, which are valued based on quoted market prices in active markets with no valuation adjustment, as Level 1 assets within the fair value hierarchy.

The Company has classified assets and liabilities measured at fair value on a recurring basis as follows (in thousands):

	Level 1	Level 2	Level 3
At December 31, 2020:			
Assets			
Cash equivalents	\$ 70,713	\$ —	\$ —
Short-term investments	225,186	—	—
Total assets	\$ 295,899	\$ —	\$ —
At December 31, 2019:			
Assets			
Cash equivalents	\$ 68,732	\$ —	\$ —
Short-term investments	37,534	—	—
Total assets	\$ 106,266	\$ —	\$ —
Liabilities			
Warrant liability	\$ —	\$ —	\$ 1,271
Total liabilities	\$ —	\$ —	\$ 1,271

As of December 31, 2019, the preferred stock warrant liability in the table above consisted of the fair value of warrants to purchase Series A-1 convertible preferred stock (“Series A-1 Preferred Stock”) and Series B convertible preferred stock (“Series B Preferred Stock”) and was based on significant inputs not observable in the market, which represent a Level 3 measurement within the fair value hierarchy. The Company’s valuation of the preferred stock warrants utilized the Black-Scholes option-pricing model, which incorporates assumptions and estimates to value the preferred stock warrants.

At the closing of the IPO in July 2020, 42,953,085 shares of outstanding Convertible Preferred Stock were automatically converted into 34,445,108 shares of common stock, and outstanding warrants to purchase an aggregate of 151,042 shares of Convertible Preferred Stock became exercisable for 121,122 shares of common stock and were reclassified into permanent equity.

As of December 31, 2019, the quantitative elements associated with the Company’s Level 3 inputs impacting the fair value measurement of the preferred stock warrant liability include the fair value per share of the underlying Series A-1 Preferred Stock and Series B Preferred Stock, the remaining contractual term of the warrants, risk-free interest rate, expected dividend yield and expected volatility of the price of the underlying preferred stock. The most significant assumption in the Black-Scholes option-pricing model impacting the fair value of the preferred stock warrants was the fair value of the Company’s convertible preferred stock as of each remeasurement date. The Company determined the fair value per share of the underlying preferred stock by taking into consideration its most recent sales of its convertible preferred stock as well as additional factors that the Company deemed relevant. As of December 31, 2019, the fair value per share of the Series A-1 Preferred Stock was \$10.36. As of December 31, 2019, the fair value per share of the Series B Preferred Stock was \$10.57. Prior to completing the IPO, the Company was a private company and lacked company-specific historical and implied volatility information of its stock. Therefore, it estimated its expected stock volatility based on the historical volatility of publicly traded peer companies for a term equal to the remaining contractual term of the warrants. The risk-free interest rate was

determined by reference to the U.S. Treasury yield curve for time periods approximately equal to the remaining contractual term of the warrants. The Company estimated a 0% dividend yield based on the expected dividend yield and the fact that the Company never paid or declared dividends. The change in fair value of warrant liability was a loss of \$0.4 million and a gain of \$0.5 million, for the years ended December 31, 2020 and 2019, respectively, included with other income (expense) within the consolidated statement of operations and comprehensive loss.

A reconciliation of the Level 3 liabilities is as follows (in thousands):

Fair value of Level 3 liabilities as of December 31, 2019	\$	1,271
Change in fair value of warrant liability		387
Reclassification of warrants to stockholders' equity (deficit)		<u>(1,658)</u>
Fair value of Level 3 liabilities as of December 31, 2020	\$	<u>—</u>

There were no transfers into or out of the Level 3 fair value hierarchy for the years ended December 31, 2020 and 2019.

Note 6. California Institute of Regenerative Medicine Awards

The Company has been awarded funding from California Institute of Regenerative Medicine (“CIRM”) to develop internal programs. Under the terms of the funding both CIRM and the Company will co-fund a specified program, under which funding is paid in developmental milestones determined as a part of the award. The Company is obligated to share future revenue for the related program with CIRM. The percentage of revenue is dependent on the amount of the award received and whether revenue is from product sales or license fees. The maximum revenue sharing amount the Company may be required to pay to CIRM is equal to nine times the total amount awarded and paid to the Company. The Company has the option to decline any and all amounts awarded by CIRM. As an alternative to revenue sharing, the Company has the option to convert the award to a loan, which such option the Company must exercise on or before ten (10) business days after the FDA notifies the Company that it has accepted the Company’s application for marketing authorization. In the event the Company exercises its right to convert the award to a loan, it would be obligated to repay the loan within ten (10) business days of making such election. Repayment amounts vary dependent on when the award is converted to a loan, ranging from 60% of the award granted to amounts received plus interest at the rate of the three-month LIBOR rate plus 10% per annum. Since the Company may be required to repay some or all of the amounts awarded by CIRM, the Company accounted for this award as a liability rather than revenue as the Company’s current intent is to convert the award into a loan. Given the uncertainty in amounts due upon repayment, the Company has recorded amounts received without any discount or interest recorded, upon determination of amounts that would become due, the Company will adjust accordingly.

In December 2017, the Company was granted an award in the amount of \$19.8 million from CIRM to support the Company’s P-BCMA-101 Phase 1 clinical trial. The award is paid based on developmental milestones, of which \$19.7 million had been received as of December 31, 2020 with up to an aggregate of \$0.1 million in future milestone payments.

In September 2018, the Company was granted an award in the amount of \$4.0 million from CIRM to support the Company’s preclinical studies for its P-PSMA-101 program. The award is paid based on developmental milestones, of which the full \$4.0 million had been received as of December 31, 2020.

Note 7. Term Debt

On July 25, 2017, the Company entered into a loan and security agreement (the “Original Loan Agreement”) with Oxford Finance LLC (“Oxford”), whereby it borrowed \$10.0 million (the “Original Term A Loan”). Balances under the Original Loan were due in monthly principal and interest payments, with a final maturity date of August 2021. The Initial Loan included a final payment fee of 8.50% of the original principal amount due upon maturity.

In August 2018, the Company entered into an Amended and Restated Loan and Security Agreement (“Amended Loan Agreement”) with Oxford, pursuant to which Oxford agreed to lend the Company up to \$30.0 million, issuable in three separate term loans of the Original Term A Loan, \$10.0 million (“New A Term Loan”), and \$10.0 million (“Term B Loan”), collectively referred to as the “Term Loans”). The Company received \$10.0 million in proceeds from the New Term A Loan, net of debt issuance costs and accrued interest of \$0.9 million. Under the terms of the Amended Loan Agreement the Company was permitted, at its sole discretion, to borrow \$10.0 million under the Term B Loan following the achievement of a defined milestone event until the earlier of 60 days thereafter or December 20, 2018. In January 2019, the Company entered into an amendment with Oxford to extend the draw period of the Term B Loan through February 15, 2019. The Company drew the remaining \$10.0 million in February 2019. In June 2020, the Company entered into an Amended and Restated Loan and Security Agreement (“2020 Amended Loan Agreement”) with Oxford. Under the terms of the 2020 Amended Loan Agreement, the interest only period on both the Term A Loan and Term B Loan was extended by 15 months, originally through September 2020 and extended through December 2021, and the final maturity date was extended by 15 months from March 1, 2023 to June 1, 2024. The 2020 Amended Loan Agreement also included a facility fee of \$0.3 million due on the amendment effective date, June 24, 2020. All other terms under the agreement remained unchanged.

The Company evaluated the amendments in accordance with ASC Topic 470 *Debt*, which requires the assessment of whether the modification was considered a substantial modification, in which case the modification would be accounted for as a debt extinguishment. Based on the Company’s evaluation, the modifications were not considered substantial and as such treated as a debt modification.

All outstanding Term Loans will mature on June 1, 2024 (the “Maturity Date”) and have interest-only payments through December 31, 2021, followed by 30 equal monthly payments of principal and unpaid accrued interest. The Term Loans bear interest at a floating per annum rate equal to (i) 6.94% plus (ii) the greater of (a) the 30-day U.S. Dollar LIBOR rate reported in The Wall Street Journal on the last business day of the month that immediately precedes the month in which the interest accrues and (b) 2.0%. The interest rate for Term Loans as of December 31, 2020 was 8.94%. The Company will be required to make a final payment of 7.5% of the principal balance outstanding, payable on the earlier of (i) the Maturity Date, (ii) acceleration of any Term Loan, or (iii) the prepayment of the Term Loans.

There is an option to prepay all, but not less than all, of the borrowed amounts, provided that the Company will be obligated to pay a prepayment fee equal to (i) 3.0% of the outstanding principal balance of the applicable Term Loan if prepayment is made after the funding date through and including the first anniversary of the funding date, (ii) 2.0% of the outstanding balance after the first anniversary through and including the second anniversary of the funding date of the Term Loan or (iii) 1.0% of the applicable Term Loan prepaid after the second anniversary of the funding date and prior to the Maturity Date.

The Company may use the proceeds from the Term Loans solely for working capital and to fund its general business requirements. The Company’s obligations under the Loan Agreement are secured by a first priority security interest in substantially all of its current and future assets, other than our intellectual property. In addition, the Company has also agreed not to encumber its intellectual property assets, except as permitted by the Loan Agreement. While any amounts are outstanding under the Loan Agreement, the Company is subject to a number of affirmative and restrictive covenants, including covenants regarding dispositions of property, business combinations or acquisitions, among other customary covenants. The Company is also restricted from paying dividends or making other distributions or payments on its capital stock in excess of \$0.3 million, on an annual basis, subject to limited exceptions. As of December 31, 2020, the Company was in compliance with all covenants under the Loan Agreement.

Pursuant to the Original Loan Agreement, on July 25, 2017, the Company issued to Oxford warrants to purchase an aggregate of up to 116,618 shares of the Company’s Series A-1 Preferred Stock (“Series A-1 Warrants”) at an exercise price of \$3.43 per share. The warrants were immediately exercisable and will expire ten years from the date of the grant. Upon the closing of the IPO these became exercisable for 93,518 shares of common stock at an exercise price of \$4.28 per share.

Pursuant to the Amended Loan Agreement, on August 13, 2018, the Company issued to Oxford warrants to purchase an aggregate of up to 17,212 shares of the Company's Series B Preferred Stock with an exercise price of \$5.81 per share ("August 2018 Series B Warrants"). On February 19, 2019, in conjunction with drawing the remaining \$10.0 million in principal, the Company issued to Oxford warrants to purchase an aggregate of up to an additional 17,212 shares of the Company's Series B Preferred Stock, with an exercise price of \$5.81 per share ("February 2019 Series B Warrants"). The August 2018 Series B Warrants and February 2019 Series B Warrants (collectively "Series B Warrants") were immediately exercisable upon issuance and will expire ten years from the date of the grant. Upon the closing of the IPO these became exercisable for 27,604 shares of common stock at an exercise price of \$7.25 per share.

The fair value of the warrants as of the issuance dates was determined to be \$0.6 million and was treated as a debt discount and as a preferred stock warrant liability. The debt discount is amortized over the term of the loan to interest expense.

As of December 31, 2020, there was \$20.0 million outstanding under the Term A Loan. The Term A Loan was recorded at its initial carrying value of \$20.0 million. In connection with the Term A Loan, the debt issuance costs of \$1.0 million have been recorded as a debt discount, including the remaining unrecognized discount from the Original Term A Loan, on the Company's consolidated balance sheets, which are being accreted to interest expense over the life of the Term A Loan. Interest on the term loan, consisting of the stated interest rate, final payment fee and amortization of the discount, is being recognized under the effective interest method using a rate of 11.28%.

As of December 31, 2020, there was \$10.0 million outstanding under the Term B Loan. The Term B Loan was recorded at its initial carrying value of \$10.0 million. In connection with the Term B Loan, the debt issuance costs of \$0.3 million have been recorded as a debt discount, on the Company's consolidated balance sheets, which are being accreted to interest expense over the life of the Term B Loan. Interest on the term loan, consisting of the stated interest rate, final payment fee and amortization of the discount, is being recognized under the effective interest method using a rate of 11.03%.

Note 8. Equity

Authorized Shares

In connection with the completion of the Company's IPO in July 2020, the Company amended its Certificate of Incorporation to authorize 250,000,000 shares of common stock, par value \$0.0001 per share, and 10,000,000 shares of undesignated preferred stock that may be issued from time to time by the Company's board of directors in one or more series, par value \$0.0001 per share.

Public Offering and Related Transaction

In July 2020, the Company completed its IPO selling 14,000,000 shares of its common stock at a public offering price of \$16.00 per share. Proceeds from the Company's IPO, net of underwriting discounts and other offering costs, were \$205.7 million. In connection with the IPO, all 42,953,085 shares of outstanding Convertible Preferred Stock were automatically converted into 34,445,108 shares of the Company's common stock. Additionally, the outstanding warrants to purchase an aggregate of 151,042 shares of Convertible Preferred Stock became exercisable for 121,122 shares of common stock and were reclassified into permanent equity.

Convertible Preferred Stock

Prior to its conversion to common stock, the Company's Convertible Preferred Stock was classified as temporary equity on the Company's balance sheets in accordance with authoritative guidance for the classification and measurement of potentially redeemable securities whose redemption is based upon certain change in control events outside of the Company's control, including liquidation, sale or transfer of control of the Company. The Company had determined not to adjust the carrying values of the convertible preferred stock to the liquidation preferences of such shares because of the uncertainty of whether or when such events would occur.

In June 2020, the Company issued and sold 10,018,300 shares of Series D Preferred Stock, at a price of \$10.93 per share, for aggregate gross proceeds of \$109.5 million.

Convertible preferred stock immediately prior to the closing of the IPO and as of December 31, 2019 consisted of the following (in thousands, except share amounts):

	July 14, 2020				
	Preferred Stock Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A Preferred Stock	9,696,798	9,696,798	\$ 31,063	\$ 31,063	7,776,095
Series A-1 Preferred Stock	3,370,263	3,253,645	11,083	11,083	2,609,176
Series B Preferred Stock	5,283,992	5,249,568	30,314	30,314	4,209,754
Series C Preferred Stock	14,734,774	14,734,774	149,713	149,713	11,816,169
Series D Preferred Stock	13,723,696	10,018,300	104,140	104,140	8,033,914
Total	<u>46,809,523</u>	<u>42,953,085</u>	<u>\$ 326,313</u>	<u>\$ 326,313</u>	<u>34,445,108</u>

	December 31, 2019				
	Preferred Stock Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A Preferred Stock	9,696,798	9,696,798	\$ 31,063	\$ 31,063	7,776,095
Series A-1 Preferred Stock	3,370,263	3,253,645	11,083	11,083	2,609,176
Series B Preferred Stock	5,283,992	5,249,568	30,314	30,314	4,209,754
Series C Preferred Stock	14,734,774	14,734,774	149,713	149,713	11,816,169
Total	<u>33,085,827</u>	<u>32,934,785</u>	<u>\$ 222,173</u>	<u>\$ 222,173</u>	<u>26,411,194</u>

Common Stock

Each share of common stock is entitled to one vote. The holders of common stock are also entitled to receive dividends whenever funds are legally available and when declared by the Company's board of directors. Since the Company's inception, there have been no dividends declared.

Common Stock Reserved for Future Issuance

Common stock reserved for future issuance consists of the following at December 31, 2020:

Stock options issued and outstanding	4,738,607
Authorized for future options and award grants	6,403,481
Authorized for future issuance under Employee Stock Purchase Plan	615,000
Total	<u>11,757,088</u>

Note 9. Stock-Based Compensation

In July 2020, the Company's board of directors and stockholders approved and adopted the 2020 Equity Incentive Plan (the "2020 Plan"). The 2020 Plan became effective as of the pricing of the IPO. Under the 2020 Plan, the Company may grant stock options, stock appreciation rights, restricted stock, restricted stock units and other stock or cash-based awards to individuals who are then employees, officers, directors or consultants of the Company. A total of 11,183,476 shares of common stock were approved to be initially reserved for issuance under the 2020 Plan. The number of shares that remain available for issuance under the 2015 Equity Incentive Plan, as amended (the "2015 Plan"), as of the effective date of the 2020 Plan and shares subject to outstanding awards under the 2015 Plan as of the effective date of the 2020 Plan that are subsequently canceled, forfeited or repurchased by the Company will be added to the shares reserved under the 2020 Plan. In addition, the number of shares of common stock available for issuance under the 2020 Plan will be automatically increased on the first day of each calendar

year during the ten-year term of the 2020 Plan, beginning with January 1, 2021 and ending with January 1, 2030, by an amount equal to 5% of the outstanding number of shares of the Company's common stock on December 31st of the preceding calendar year or such lesser amount as determined by the Company's board of directors.

As of December 31, 2020, there were 6,403,481 shares available for future option grants or direct issuance under the 2020 Plan. To date, the Company has issued ISOs and NSOs. Shares issued under the 2020 Plan are newly issued shares and there is no intention to repurchase previously issued shares. The exercise price of options granted under the 2020 Plan cannot be less than 100% of the fair value of the common stock. The term and vesting period of each option shall be stated in the underlying agreements. However, the term shall be no more than ten years from the date of grant and the Company's normal practice is generally a vesting period over four years. In the case of an ISO granted to an optionee who, at the time the option is granted, owns stock representing more than ten percent of the voting power of all classes of stock of the Company, the term of the option shall be five years from the date of grant and issued at 110% of the fair value at the date of grant.

Following is a summary of the Company's stock option plan activity and related information for the year ended December 31, 2020:

	Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (Years)	Intrinsic Value (thousands)
Balance at January 1, 2020	3,611,030	\$ 8.58	8.65	
Options Granted	1,510,762	13.36		
Exercised	(219,425)	1.02		
Cancelled	(163,760)	11.76		
Balance at December 31, 2020	<u>4,738,607</u>	\$ 10.34	8.34	\$ 10,017
Options Vested & Expected to Vest as of December 31, 2020	4,738,607	\$ 10.34	8.34	\$ 10,017
Options Exercisable as of December 31, 2020	1,886,521	\$ 7.56	6.84	\$ 8,226

All stock option shares and exercise prices were adjusted retrospectively for the Company's reverse stock split, which became effective in July 2020.

The aggregate intrinsic value of options exercised during the years ended December 31, 2020 and 2019 was \$2.4 million and \$0.7 million, respectively, determined as of the date of exercise. The Company received \$0.2 million and \$0.1 million in cash from options exercised during the years ended December 31, 2020 and 2019, respectively.

The Company recorded stock-based compensation expense in the following expense categories of its consolidated statements of operations (in thousands):

	Year Ended December 31,	
	2020	2019
Research and development	\$ 3,872	\$ 1,703
General and administrative	3,348	1,347
Total stock-based compensation	<u>\$ 7,220</u>	<u>\$ 3,050</u>

As of December 31, 2020, total unrecognized compensation cost related to stock options was \$23.0 million, and the weighted-average period over which this cost is expected to be recognized is approximately 3.0 years.

The weighted-average fair value of options granted during the years ended December 31, 2020 and 2019 was \$8.65 and \$8.47 per share, respectively. Total fair value of shares vested during the years ended December 31, 2020 and 2019 was \$6.9 million and \$2.8 million, respectively.

The assumptions that the Company used to determine the fair value of options granted to employees, non-employees and directors were as follows:

	Year Ended December 31,	
	2020	2019
Risk-free interest rate	0.4%-1.4%	1.6%-2.6%
Expected volatility	79-86%	80-87%
Expected term (years)	5.5-6	4-6
Dividend Yield	—	—

Risk-free interest rate—The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of option.

Expected volatility—The expected volatility is estimated based on the average volatility for comparable publicly traded biotechnology companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on their similar size, stage in the life cycle or area of specialty.

Expected term—The expected term represents the period that stock-based awards are expected to be outstanding. The expected term for option grants is determined using the simplified method which is used when there is insufficient historical data about exercise patterns and post-vesting employment termination behavior. The simplified method deems the expected term to be the midpoint between the vesting date and the contractual life of the stock-based awards.

Expected dividend—The Company has never paid dividends on its common stock, and has no plans to pay any dividends on its common stock. Therefore, the Company used an expected dividend yield of zero.

In July 2020, the Company’s board of directors and stockholders approved and adopted the 2020 Employee Stock Purchase Plan (the “ESPP”). The ESPP became effective as of the pricing of the IPO. A total of 615,000 shares of common stock were approved to be initially reserved for issuance under the ESPP. In addition, the number of shares of common stock available for issuance under the ESPP will be automatically increased on the first day of each calendar year during the first ten-years of the term of the ESPP, beginning with January 1, 2021 and ending with January 1, 2030, by an amount equal to the lesser of (i) 1% of the outstanding number of shares of the Company’s common stock on December 31st of the preceding calendar year, (ii) 1,230,000 shares of common stock or (iii) such lesser amount as determined by the Company’s board of directors.

Note 10. Commitments and Contingencies

Operating Leases

As of December 31, 2020, the Company had operating leases for manufacturing, laboratory and office space in San Diego, California consisting of approximately 83,000 square feet with remaining lease terms of 108 months. The manufacturing and laboratory and office space lease agreements include two options to extend the term for a period of 5 years each. Additionally, the Company had operating leases for dedicated manufacturing suites at its contract manufacturers with remaining lease terms of up to 12 months.

In March 2016, the Company entered into a lease agreement for a facility in San Diego, California to be used for research and development and administrative activities. The lease commenced on June 30, 2016 and had a 10.5-year initial term. The lease provided for rent abatements and scheduled increases in base rent. In connection with the lease, the Company made a one-time cash security deposit in the amount of \$0.1 million, included in other long-term assets in the Company’s consolidated balance sheet. As a result of outgrowing the office space and lab space and having executed a lease agreement for a larger facility, the Company terminated this lease agreement effective April 30, 2019. As part of the lease termination agreement, the Company committed to pay a \$1.5 million cancellation fee to the landlord in three installments over a 1.5-year period. As of December 31, 2019, the Company recorded the lease cancellation fee, gain from the deferred rent write-off, and abandonment of the fixed assets as part of loss from continuing operations. As of December 31, 2020, there were no further obligations related to this lease.

In October 2018, the Company entered into a lease agreement for a facility in San Diego, California to be used for research and development and administrative activities. The lease term commenced on April 1, 2019 and will expire on December 31, 2029. The lease provided for tenant improvements of \$4.2 million and as costs were incurred, the Company performed an analysis to determine treatment based on the type of leasehold improvement. Assets determined to be lessee assets were not material and were recorded on the consolidated balance sheet as an asset with a corresponding liability within the lease liability. As of December 31, 2019, all costs were incurred, and construction was completed.

In October 2019, the Company entered into a lease amendment to expand the existing premises. The lease term for the additional premises commenced on July 29, 2020 and will expire on December 31, 2029. The lease amendment provided for additional tenant reimbursements of \$1.5 million and as costs were incurred, the Company performed additional analysis to determine treatment based on the type of leasehold improvement. As of December 31, 2020, all costs were incurred, and construction was completed. Both the original lease and amendment provides for rent abatements and scheduled increases in base rent. In connection with the lease and its amendment, the Company made cash security deposits in the amount of \$0.3 million, included in other long-term assets in the Company's consolidated balance sheet. As of December 31, 2020, the Company held \$0.1 million as a receivable within other current assets for amounts to be reimbursed by the landlord.

In July 2019, the Company entered into a lease agreement for a facility in San Diego, California to be retrofitted to Good Manufacturing Practice standards and plans to use the facility for manufacturing in its early-stage clinical trials. The lease term commenced on June 26, 2020 and will expire on December 31, 2029. The lease provided for tenant improvements of \$2.9 million and as costs were incurred, the Company performed analysis to determine treatment based on the type of leasehold improvement. As of December 31, 2020, all costs were incurred, and construction was completed. The lease provides for rent abatements and scheduled increases in base rent. In connection with the lease, the Company made a one-time cash security deposit in the amount of \$0.1 million, included in other long-term assets in the Company's consolidated balance sheet.

On January 1, 2020, on the adoption of ASC 842, the Company recognized initial lease receivables of \$2.7 million, ROU lease assets of \$22.3 million, which was adjusted for the deferred rent balance of \$2.3 million and an initial lease liability of \$27.3 million, with respect to the existing leases. The leases in San Diego include an option to extend, which was not recognized as part of the lease liability and ROU lease assets as the Company was not reasonably certain it would exercise the extension right. Under ASC 840, the Company had been the deemed owner under construction of the manufacturing facility. Upon the adoption of ASC 842 the Company derecognized the amounts previously presented on its balance sheet related to its manufacturing facility including construction in progress of \$2.1 million within property and equipment, and the construction financing obligation of \$2.5 million recorded within other long-term liabilities and \$0.3 million of other receivables within prepaid and other current assets as of December 31, 2019. The Company also recorded a cumulative adjustment to the opening balance of accumulated deficit of \$0.1 million.

Upon adoption of ASC 842, the Company determined the lease would be accounted for as an operating lease. Further, upon adoption of ASC 842, the Company determined it was the owner of the tenant improvements but did not control the construction project and therefore the fair value of the building was derecognized and costs incurred by the Company related to the tenant improvements of \$13.3 million were recorded as leasehold improvements in property and equipment, net on the consolidated balance sheet as of December 31, 2020 and will be depreciated over the remaining lease term.

During the twelve months ended December 31, 2020 and 2019, the Company recognized \$6.0 million and \$2.5 million, respectively, of operating lease expense. The Company recognized an immaterial amount of variable operating lease expense for the twelve months ended December 31, 2020. During the twelve months ended December 31, 2020, the Company paid \$4.8 million in cash payments for its operating leases. As of December 31, 2020, the weighted average remaining lease term for operating leases was 8.7 years. As of December 31, 2020 the weighted-average discount rate for operating leases was and 8.9%.

As of December 31, 2020, maturities of lease liabilities were as follows (in thousands):

Year ending December 31,	
2021	\$ 5,225
2022	4,333
2023	4,463
2024	4,597
2025	4,735
Thereafter	20,403
Total lease payments	43,756
Imputed interest	(13,574)
Total lease liability	30,182
Less current portion of lease liability	4,808
Lease liability, net of current portion	\$ 25,374

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, contract research organizations, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors and certain of its executive officers that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. The Company has not incurred any material costs as a result of such indemnifications and is not currently aware of any indemnification claims.

License Agreement with Janssen Biotech Inc.

On August 3, 2015, the Company entered into a license agreement (“Janssen Agreement”) with Janssen pursuant to which the Company obtained exclusive worldwide rights to research, develop, manufacture and commercialize pharmaceutical products comprising autologous CAR-modified T-cells or any CAR-modified natural killer or CAR-modified natural-killer-like cells expressing certain Centyrin molecules or Centyrin CAR molecules for the treatment or prevention of any disease in humans. Pursuant to the Janssen Agreement, the Company paid Janssen an upfront fee of \$0.2 million. Based on milestone developments, the Company has paid an additional \$4.0 million through December 31, 2020. The Company is required to pay Janssen up to an aggregate of \$75.8 million upon the achievement of certain clinical, regulatory and sales milestones for the first licensed product and up to an aggregate of \$46.8 million upon the achievement of certain clinical, regulatory and sales milestones for each licensed product thereafter. The Company is also obligated to pay, on a product-by-product and country-by-country basis, royalties in the low single-digit percentage range on annual net sales.

April 2017 Commercial License Agreement with TeneoBio, Inc.

On April 27, 2017, the Company entered into a commercial license agreement (the “2017 TeneoBio Agreement”) with TeneoBio, Inc. (“TeneoBio”) pursuant to which the Company obtained exclusive worldwide rights to use and develop pharmaceutical products comprising allogeneic T-cells expressing a CAR molecule containing certain heavy chain sequences provided by TeneoBio.

Pursuant to the 2017 TeneoBio Agreement, the Company has paid TeneoBio \$0.5 million through the Company’s selection of the antibodies licensed under the 2017 TeneoBio Agreement. The Company is required to pay TeneoBio up to an aggregate of \$20.5 million upon the first achievement of certain clinical and regulatory milestones for any allogeneic product and up to an aggregate of \$20.5 million upon the first achievement of certain clinical and regulatory milestones for any autologous product. The Company is also obligated to pay, on a product-by-product and country-by-country basis, a royalty in the low single-digit percentage on net sales of any licensed products.

August 2018 Commercial License Agreement with TeneoBio, Inc.

On August 3, 2018, the Company entered into a commercial license agreement (the “2018 TeneoBio Agreement”) with TeneoBio pursuant to which the Company obtained exclusive rights to research, develop and commercialize up to a certain number of targets from TeneoBio. Pursuant to the 2018 TeneoBio Agreement, the Company has paid TeneoBio an upfront fee of \$4.0 million. The Company is required to pay TeneoBio up to an aggregate of \$31.0 million upon the first achievement of certain clinical and regulatory milestones for each product, none of which have been met. The Company is also obligated to pay, on a product-by-product and country-by-country basis, a royalty in the low single-digit percentage on net sales of any licensed products.

October 2019 License Agreement with Genus Oncology, LLC

On October 24, 2019, the Company entered into a license agreement (the “Genus Agreement”) with Genus Oncology, LLC (“Genus”), pursuant to which the Company paid Genus an upfront fee of \$1.5 million and Genus granted the Company the option, which is exercisable for an additional \$1.5 million fee, to obtain an exclusive worldwide license to research, develop and commercialize pharmaceutical products incorporating CAR cells expressing antibodies and derivatives thereof targeting MUC1.

Pursuant to the Genus Agreement, the Company is also required to pay Genus up to an aggregate of \$71.0 million upon first achievement of certain clinical, regulatory and sales milestones for any Genus licensed product and companion diagnostics. The Company is also obligated to pay, on a product-by-product and country-by-country basis, tiered royalties in the low to mid-single-digit percentage on net sales of any Genus licensed products and related companion diagnostics.

Legal Contingencies

In the ordinary course of business, the Company may face claims brought by third parties against the Company. The Company does not believe that there is any litigation, asserted or unasserted claim pending that could, individually or in the aggregate, have a material adverse effect on the Company’s results of operations or financial condition.

Note 11. Income Taxes

The components of the pretax loss from operations were all attributed to the United States. There was no income tax expense or benefit for the years ended December 31, 2020 and 2019.

The (benefit from) provision for income taxes differs from the amount of income tax determined by applying the applicable U.S. statutory federal income tax rate to pretax income as a result of the following differences as of (in thousands):

	<u>Year Ended December 31,</u>	
	<u>2020</u>	<u>2019</u>
Federal statutory rate	\$ (27,253)	\$ (18,170)
Adjustments for tax effects of:		
State taxes, net	(8,581)	(5,384)
Permanent adjustments	134	(43)
Contingent consideration	—	1,403
Stock-based compensation	956	428
Tax credits	(10,735)	(5,828)
Unrecognized tax benefits	2,445	1,643
Other, net	(27)	55
Change in valuation allowance	43,061	25,896
Total	<u>\$ —</u>	<u>\$ —</u>

Significant components of the Company's deferred tax assets and liabilities consist of the following as of (in thousands):

	December 31,	
	2020	2019
Deferred tax assets:		
Amortization	\$ 1,558	\$ 1,706
Grant income	6,648	5,483
Accrued expenses	1,855	915
Net operating losses	65,058	33,279
Income tax credit carryforwards	15,727	7,101
Lease liabilities	8,388	—
Other, net	982	1,151
Total deferred tax assets	100,216	49,635
Deferred tax liabilities:		
Depreciation	(1,877)	(1,295)
Acquired indefinite lived intangibles	(369)	(369)
Right of use assets	(6,934)	—
Other, net	—	—
Total deferred tax liabilities	(9,180)	(1,664)
Valuation allowance	(91,091)	(48,026)
Net deferred tax liability	\$ (55)	\$ (55)

The realization of deferred tax assets may be dependent on the Company's ability to generate sufficient income in future years in the associated jurisdiction to which the deferred tax assets relate. The Company considers all available positive and negative evidence, including scheduled reversals of deferred tax liabilities, projected future taxable income, tax planning strategies, and recent financial performance. A valuation allowance of \$91.1 million has been recorded as of December 31, 2020, as compared to \$48.0 million, as of December 31, 2019. The valuation allowance is based on management's assessment that it is more likely than not that the Company will not have taxable income in the foreseeable future.

Deferred tax liabilities associated with indefinite-life intangibles cannot be considered a source of income to support the realization of deferred tax assets because the reversal of these deferred tax liabilities is considered indefinite. However, as the Company has an indefinite-life asset with an unlimited loss carryforward period within the same jurisdiction, and of appropriate character, the deferred tax liability associated with the indefinite-life intangible constitutes a source of taxable income to support the realization of the deferred tax asset, since both have indefinite reversal or expiration periods.

As of December 31, 2020, the Company had federal and state net operating loss carryforwards of \$23.3 million and \$236.4 million, respectively, which begin to expire in 2032 and the Company had federal net operating loss carryforwards that do not expire but utilization is limited to 80% of taxable income for any given tax year in the amount of \$205.4 million.

As of December 31, 2020, the Company had federal orphan drug credits and research and development credits and state research and development tax credits of \$17.7 million and \$4.1 million, respectively. The federal research and development tax credits will begin to expire in 2032, while the state credits do not expire.

Additionally, the utilization of the net operating loss and research and development tax credit carryforwards is subject to an annual limitation under Section 382 of the Internal Revenue Code. Future ownership changes as determined under Section 382 could further limit the utilization of net operating loss carryforwards. Due to the existence of the valuation allowance, future changes in the deferred tax assets related to these tax attributes will not impact the Company's effective tax rate.

The Company is subject to taxation in the U.S. and state jurisdictions. As of December 31, 2020, the Company's tax years beginning 2012 to date are subject to examination by federal and other state taxing authorities

due to the carry forward of unutilized net operating losses and research and development tax credits. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service or state tax authorities to the extent utilized in a future period. The Company is not currently under examination by the IRS or state and local tax authorities.

As of December 31, 2020, the Company had unrecognized tax benefits of \$5.5 million, determined as follows:

	December 31,	
	2020	2019
Balance at beginning of the year	\$ 2,470	\$ 755
Increase for current year positions	3,211	1,715
Increase for prior year positions	(224)	—
Balance at the end of year	<u>\$ 5,457</u>	<u>\$ 2,470</u>

These unrecognized tax benefits are not expected to change within the next twelve months. Of the \$5.5 million of unrecognized tax benefits, zero would impact the effective tax rate due to the valuation allowance, if reversed. The Company recognizes interest and penalties related to uncertain tax positions as a component of income tax expense. As of December 31, 2020, there are no accrued interest or penalties.

Note 12. Employee Benefit Plan

In 2015, the Company adopted a defined contribution retirement savings plan under Section 401(k) of the Internal Revenue Code. This plan covers all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Company contributions to the plan may be made at the discretion of the Board of Directors. Total contributions by the Company during the years ended December 31, 2020 and 2019 were \$0.6 million and \$0.3 million, respectively.

Note 13. Net Loss Per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share amounts):

	Year Ended December 31,	
	2020	2019
Numerator:		
Net loss	\$ (129,775)	\$ (86,527)
Net loss attributable to common stockholders	<u>\$ (129,775)</u>	<u>\$ (86,527)</u>
Denominator:		
Weighted-average common stock outstanding, basic and diluted	35,996,901	12,618,413
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (3.61)</u>	<u>\$ (6.86)</u>

The Company's potentially dilutive securities, which include warrants to purchase common stock and common stock options, have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share. Therefore, the weighted-average number of shares of common stock outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential common shares, presented based on amounts outstanding at each period

end, from the computation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	<u>Year Ended December 31,</u>	
	<u>2020</u>	<u>2019</u>
Convertible preferred stock (as converted to common stock)	—	26,411,194
Warrants to purchase convertible preferred stock (as converted to common stock)	121,122	121,122
Stock options to purchase common stock	<u>4,738,607</u>	<u>3,610,779</u>
	<u>4,859,729</u>	<u>30,143,095</u>

Item 16. Form 10-K Summary.

None.

Exhibit Index

Exhibit Number	Description
2.1^	<u>Agreement and Plan of Merger and Reorganization, by and among the Registrant, Hermes Merger Sub I, Inc., Hermes Merger Sub II, LLC, Vindico NanoBioTechnology, Inc. and Christopher Young as Stockholders' Representative, dated October 10, 2016, as amended (incorporated by reference to Exhibit 2.1 to the Registrant's Registration Statement on Form S-1 (File No. 333-239321), filed with the SEC on June 19, 2020).</u>
3.1	<u>Amended and Restated Certificate of Incorporation, of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K (File No. 001-39376), filed with the SEC on July 14, 2020).</u>
3.2	<u>Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K (File No. 001-39376), filed with the SEC on July 14, 2020).</u>
4.1	<u>Form of Common Stock Certificate of the Registrant (incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1 (File No. 333-239321), filed with the SEC on June 19, 2020).</u>
4.2^	<u>Amended and Restated Investors' Rights Agreement, by and among the Registrant and certain of its stockholders, dated June 24, 2020 (incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-239321), filed with the SEC on July 6, 2020).</u>
4.3	<u>Form of Warrant issued to Oxford Finance LLC, dated July 25, 2017 (incorporated by reference to Exhibit 4.3 to the Registrant's Registration Statement on Form S-1 (File No. 333-239321), filed with the SEC on June 19, 2020).</u>
4.4	<u>Form of Warrant issued to Oxford Finance LLC, dated August 13, 2018 (incorporated by reference to Exhibit 4.4 to the Registrant's Registration Statement on Form S-1 (File No. 333-239321), filed with the SEC on June 19, 2020).</u>
4.5	<u>Form of Warrant issued to Oxford Finance LLC, dated February 11, 2019 (incorporated by reference to Exhibit 4.5 to the Registrant's Registration Statement on Form S-1 (File No. 333-239321), filed with the SEC on June 19, 2020).</u>
4.6	<u>Description of Common Stock.</u>
10.1+	<u>Form of Indemnity Agreement, by and between the Registrant and its directors and officers (incorporated by reference to Exhibit 10.1 to the Registrant's Registration Statement on Form S-1 (File No. 333-239321), filed with the SEC on June 19, 2020).</u>
10.2+	<u>Poseida Therapeutics, Inc. 2015 Equity Incentive Plan, as amended, and Forms of Option Grant Notice, Option Agreement and Notice of Exercise thereunder (incorporated by reference to Exhibit 10.2 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-239321), filed with the SEC on July 6, 2020).</u>
10.3+	<u>Poseida Therapeutics, Inc. 2020 Equity Incentive Plan and Forms of Stock Option Grant Notice, Option Agreement and Notice of Exercise thereunder (incorporated by reference to Exhibit 10.3 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-239321), filed with the SEC on July 6, 2020).</u>
10.4+	<u>Poseida Therapeutics, Inc. 2020 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.4 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-239321), filed with the SEC on July 6, 2020).</u>

- 10.5+ [Poseida Therapeutics, Inc. Severance and Change in Control Plan and Form of Participation Agreement \(incorporated by reference to Exhibit 10.5 to the Registrant's Registration Statement on Form S-1, as amended \(File No. 333-239321\), filed with the SEC on July 6, 2020\).](#)
- 10.6+^ [Executive Employment Agreement, by and between the Registrant and Eric Ostertag, dated June 1, 2015 \(incorporated by reference to Exhibit 10.6 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)
- 10.7+^ [Executive Employment Agreement, by and between the Registrant and Mark Gergen, dated February 19, 2018 \(incorporated by reference to Exhibit 10.7 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)
- 10.8+^ [Offer Letter, by and between the Registrant and Matthew Spear, dated June 13, 2016 \(incorporated by reference to Exhibit 10.8 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)
- 10.9+^ [Offer Letter, by and between the Registrant and Kerry Ingalls, dated July 29, 2019 \(incorporated by reference to Exhibit 10.9 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)
- 10.10+^ [Offer Letter, by and between the Registrant and Johanna Mylet, dated June 8, 2015 \(incorporated by reference to Exhibit 10.10 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)
- 10.11+ [Poseida Therapeutics, Inc. Non-Employee Director Compensation Policy \(incorporated by reference to Exhibit 10.11 to the Registrant's Registration Statement on Form S-1, as amended \(File No. 333-239321\), filed with the SEC on July 6, 2020\).](#)
- 10.12* [License Agreement, by and between the Registrant and Janssen Biotech, Inc., effective August 3, 2015 \(incorporated by reference to Exhibit 10.12 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)
- 10.13* [Commercial License Agreement, by and between the Registrant and TeneoBio, Inc., effective April 27, 2017 \(incorporated by reference to Exhibit 10.13 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)
- 10.14* [Commercial License Agreement, by and between the Registrant and TeneoBio, Inc., effective August 3, 2018 \(incorporated by reference to Exhibit 10.14 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)
- 10.15* [License Agreement, by and between the Registrant and Helmholtz-Zentrum München—Deutsches Forschungszentrum für Gesundheit und Umwelt GmbH, dated May 20, 2016 \(incorporated by reference to Exhibit 10.15 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)
- 10.16* [License Agreement, by and between the Registrant and Genus Oncology, LLC, effective October 24, 2019 \(incorporated by reference to Exhibit 10.16 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)
- 10.17^ [Loan and Security Agreement, by and among the Registrant, Vindico NanoBioTechnology, LLC and Oxford Finance LLC, dated July 25, 2017, as amended on May 15, 2018, August 13, 2018, January 3, 2019, and June 24, 2020 \(incorporated by reference to Exhibit 10.17 to the Registrant's Registration Statement on Form S-1, as amended \(File No. 333-239321\), filed with the SEC on July 6, 2020\).](#)
- 10.18 [Lease, by and between the Registrant and BMR-9360-9390 Towne Centre LP, dated October 1, 2018, as amended on October 4, 2019 and March 11, 2020 \(incorporated by reference to Exhibit 10.18 to the Registrant's Registration Statement on Form S-1 \(File No. 333-239321\), filed with the SEC on June 19, 2020\).](#)

10.19	<u>Lease, by and between the Registrant and BMR-Eastgate Mall LP, dated July 12, 2019 (incorporated by reference to Exhibit 10.19 to the Registrant's Registration Statement on Form S-1 (File No. 333-239321), filed with the SEC on June 19, 2020).</u>
21.1	<u>Subsidiaries of the Registrant.</u>
23.1	<u>Consent of Independent Registered Public Accounting Firm.</u>
24.1	<u>Power of Attorney (reference is made to the signature page hereto).</u>
31.1	<u>Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) Under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u>
31.2	<u>Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) Under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u>
32.1	<u>Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u>
32.2	<u>Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u>
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

^ Certain exhibits and schedules have been omitted pursuant to Item 601(a)(5) of Regulation S-K. The Registrant hereby undertakes to furnish supplementally a copy of any omitted exhibit or schedule upon request by the SEC.

+ Indicates management contract or compensatory plan.

* Pursuant to Item 601(b)(10) of Regulation S-K, certain portions of this exhibit have been omitted by means of marking such portions with asterisks because the Registrant has determined that the information is not material and would likely cause competitive harm to the Registrant if publicly disclosed.

DESCRIPTION OF COMMON STOCK

The following summary description of the common stock of Poseida Therapeutics, Inc. (we, our or us) is based on the provisions of our amended and restated certificate of incorporation, or Restated Certificate, as well as our amended and restated bylaws, or Restated Bylaws, and the applicable provisions of the Delaware General Corporation Law. This information is qualified entirely by reference to the applicable provisions of our Restated Certificate, Restated Bylaws, and the Delaware General Corporation Law. Our Restated Certificate and Restated Bylaws have previously been filed as exhibits with the Securities and Exchange Commission.

General

Our authorized capital stock consists of 250,000,000 shares of common stock, par value \$0.0001 per share, and 10,000,000 shares of preferred stock, par value \$0.0001 per share. Our board of directors has the authority, without stockholder approval, except as required by the listing standards of The Nasdaq Stock Market LLC, to issue additional shares of our capital stock.

Dividend Rights

Subject to preferences that may apply to shares of preferred stock outstanding at the time, the holders of outstanding shares of our common stock are entitled to receive dividends out of funds legally available if our board of directors, in its discretion, determines to issue dividends and only then at the times and in the amounts that our board of directors may determine.

Voting Rights

The holders of our common stock are entitled to one vote per share. Our stockholders do not have the ability to cumulate votes for the election of directors. Our Restated Certificate and Restated Bylaws provide for a classified board of directors consisting of three classes of approximately equal size, each serving staggered three-year terms. Only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms.

No Preemptive or Similar Rights

Our common stock is not entitled to preemptive rights and is not subject to conversion, redemption or sinking fund provisions.

Right to Receive Liquidation Distributions

Upon our dissolution, liquidation or winding-up, the assets legally available for distribution to our stockholders are distributable ratably among the holders of our common stock, subject to prior satisfaction of all outstanding debt and liabilities and the preferential rights and payment of liquidation preferences, if any, on any outstanding shares of preferred stock.

Anti-Takeover Provisions

Delaware Law

We are governed by the provisions of Section 203 of the Delaware General Corporation Law regulating corporate takeovers. This section prevents some Delaware corporations from engaging, under some circumstances, in a business combination, which includes a merger or sale of at least 10% of the corporation's assets with any interested stockholder, meaning a stockholder who, together with affiliates and associates, owns or, within three years prior to the determination of interested stockholder status, did own 15% or more of the corporation's outstanding voting stock, unless:

- the transaction is approved by the board of directors prior to the time that the interested stockholder became an interested stockholder;
- upon closing of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction began, excluding for purposes of determining the voting stock outstanding those shares owned (i) by persons who are directors and also officers and (ii) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- subsequent to such time that the stockholder became an interested stockholder the business combination is approved by the board of directors and authorized at an annual or special meeting of stockholders by at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

A Delaware corporation may "opt out" of these provisions with an express provision in its original certificate of incorporation or an express provision in its certificate of incorporation or amended and restated bylaws resulting from a stockholders' amendment approved by at least a majority of the outstanding voting shares. We have not opted out of these provisions. As a result, mergers or other takeover or change in control attempts of us may be discouraged or prevented.

Restated Certificate and Restated Bylaws Provisions

Our Restated Certificate and Restated Bylaws include a number of provisions that may have the effect of deterring hostile takeovers or delaying or preventing changes in control of our management team, including the following:

- *Board of Directors Vacancies.* Our Restated Certificate and Restated Bylaws authorize our board of directors to fill vacant directorships, including newly-created seats. In addition, the number of directors constituting our board of directors shall be set only by resolution adopted by a majority vote of our entire board of directors. These provisions may prevent a stockholder from increasing the size of our board of directors and gaining control of our board of directors by filling the resulting vacancies with its own nominees.
- *Classified Board.* Our Restated Certificate and Restated Bylaws provide that our board of directors will be classified into three classes of directors, each of which will hold office for a three-year term. In addition, directors may only be removed from the board of directors for cause and only by the approval of 66-2/3% of our then-outstanding shares of our common stock. A third party may be discouraged from making a tender offer or otherwise attempting to obtain control of us as it is more difficult and time consuming for stockholders to replace a majority of the directors on a classified board of directors.

- *Stockholder Action; Special Meeting of Stockholders.* Our Restated Certificate provides that stockholders are not able to take action by written consent and are only able to take action at annual or special meetings of our stockholders. Stockholders are not permitted to cumulate their votes for the election of directors. Our Restated Bylaws further provide that special meetings of our stockholders may be called only by a majority vote of our entire board of directors, the chairman of our board of directors or our chief executive officer.
- *Advance Notice Requirements for Stockholder Proposals and Director Nominations.* Our Restated Bylaws provide advance notice procedures for stockholders seeking to bring business before our annual meeting of stockholders, or to nominate candidates for election as directors at any meeting of stockholders. Our Restated Bylaws also specify certain requirements regarding the form and content of a stockholder's notice. These provisions may make it more difficult for our stockholders to bring matters before our annual meeting of stockholders or to make nominations for directors at our meetings of stockholders.
- *Issuance of Undesignated Preferred Stock.* Our board of directors has the authority, without further action by the holders of common stock, to issue up to 10,000,000 shares of undesignated preferred stock with rights and preferences, including voting rights, designated from time to time by our board of directors. The existence of authorized but unissued shares of preferred stock will enable our board of directors to render more difficult or discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise.

Choice of Forum

Our Restated Certificate provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (1) any derivative action or proceeding brought on our behalf; (2) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders; (3) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the Delaware General Corporation Law, our Restated Certificate or our Restated Bylaws; (4) any action or proceeding to interpret, apply, enforce or determine the validity of our Restated Certificate or our Restated Bylaws; (5) any action or proceeding as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and (6) any action asserting a claim against us or any of our directors, officers or other employees, governed by the internal affairs doctrine; provided, that, this provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act, or any other claim for which the federal courts have exclusive jurisdiction. Our Restated Certificate further provides that the federal district courts of the United States of America shall be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, unless we consent in writing to the selection of an alternative forum. These choice of forum provisions may limit a stockholder's ability to bring certain claims in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, other employees or stockholders, which may discourage lawsuits with respect to such claims, although our stockholders will not be deemed to have waived our compliance with federal securities laws and the rules and regulations thereunder.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A.

Listing

Our common stock is listed on The Nasdaq Global Select Market under the symbol “PSTX.”

245384150 v3

Subsidiaries of Poseida Therapeutics, Inc.:

<u>NAME</u>	<u>JURISDICTION OF INCORPORATION</u>
Vindico NanoBioTechnology, LLC	Delaware

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the incorporation by reference in the Registration Statement on Form S-8 (No. 333-240048) of Poseida Therapeutics, Inc. of our report dated March 11, 2021 relating to the financial statements, which appears in this Form 10-K.

/s/ PricewaterhouseCoopers LLP
San Diego, California
March 11, 2021

**CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Eric Ostertag, M.D., Ph.D., certify that:

1. I have reviewed this Annual Report on Form 10-K of Poseida Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 11, 2021

By: _____ /s/ Eric Ostertag
Eric Ostertag, M.D., Ph.D.
Chief Executive Officer
(Principal Executive Officer)

**CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Mark J. Gergen, J.D., certify that:

1. I have reviewed this Annual Report on Form 10-K of Poseida Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 11, 2021

By: _____ /s/ Mark J. Gergen
Mark J. Gergen, J.D.
President, Chief Business Officer
(Principal Financial Officer)

**CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report on Form 10-K of Poseida Therapeutics, Inc. (the "Company") for the year ending December 31, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I hereby certify to the best of my knowledge, pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that:

- (1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Exchange Act; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 11, 2021

By: _____ /s/ Eric Ostertag

Eric Ostertag, M.D., Ph.D.
Chief Executive Officer
(Principal Executive Officer)

This certification accompanies the Report to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.

**CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report on Form 10-K of Poseida Therapeutics, Inc. (the "Company") for the year ending December 31, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I hereby certify to the best of my knowledge, pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that:

- (1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Exchange Act; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 11, 2021

By: _____ /s/ Mark J. Gergen

Mark J. Gergen, J.D.
President, Chief Business Officer
(Principal Financial Officer)

This certification accompanies the Report to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.