

## Risk Factors Comparison 2025-03-20 to 2024-03-20 Form: 10-K

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The following risk factors and other information included in this Annual Report on Form 10- K (“ Annual Report ”), including our financial statements and related notes thereto, should be carefully considered. The risks and uncertainties described below are not the only risks and uncertainties we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Please see the discussion regarding some of the forward- looking statements that are qualified by these risk factors contained elsewhere in this Annual Report. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

**Risks Related to Our Financial Position and Need for Additional Capital** We have incurred significant net losses since inception. We expect to incur net losses for the foreseeable future and may never achieve or maintain profitability. Since inception, we have not generated any revenue and have incurred significant operating losses. For the years ended December 31, **2024 and 2023** ~~and 2022~~, our net loss was \$ **117.116**. 9 million and \$ **92.117**. ~~19~~ million, respectively. As of December 31, **2023-2024**, we had an accumulated deficit of \$ **340.457**. ~~10~~ million. We have financed our operations primarily through the sale of our capital stock. We have devoted all of our efforts to organizing and staffing our company, business and scientific planning, raising capital, acquiring and developing technology, identifying potential product candidates, undertaking studies of potential product candidates, developing manufacturing capabilities and evaluating a clinical path for our pipeline programs. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if and as we: • advance and complete clinical trials of our product candidates, including trem- cel and **VCAR33 (previously called VCAR33ALLO)**; • initiate clinical development of other product candidates; • continue our current research programs and development of other potential product candidates from our current research programs; • seek to identify additional product candidates and research programs; • initiate preclinical testing and clinical trials for any other product candidates we identify and develop; • maintain, expand, enforce, defend and protect our intellectual property portfolio and provide reimbursement of third- party expenses related to our patent portfolio; • research, develop, acquire or in- license additional targeted therapies that could potentially be used in combination or sequence with trem- cel or other engineered hematopoietic stem cell (“ eHSC ”) product candidates; • seek marketing approvals for any product candidates that successfully complete clinical trials; • ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval; • adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products; • further develop our genome engineering capabilities; • hire additional research and development and clinical personnel; • hire commercial personnel and advance market access and reimbursement strategies; • add operational, financial and management information systems and personnel, including personnel to support our product development; • acquire or in- license product candidates, intellectual property and technologies; • develop or in- license manufacturing and distribution technologies; • maintain and expand our own current Good Manufacturing Practices (“ cGMP ”) manufacturing facility; • should we decide to do so and receive approval for any of our product candidates, build and maintain, or purchase and validate, commercial- scale manufacturing facilities designed to comply with cGMP requirements; and • operate as a public company. We have not completed clinical development of any product candidate and expect that it will be several years, if ever, before we have a product candidate ready for commercialization. To become and remain profitable, we must develop and, either directly or through collaborators, eventually commercialize a product or products with significant market potential. This will require us to be successful in a range of challenging activities, including identifying product candidates, completing preclinical testing and clinical trials of 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 do, may never generate revenues that are significant or large enough to achieve profitability. Our product candidates and research programs are currently only in the early stages of development. Because of the numerous risks and uncertainties associated with developing product candidates, we are unable to predict the extent of any future losses or when we will become profitable, if at all. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. 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 our stockholders to lose all or part of their investments in us.

**There is substantial doubt regarding our ability to continue as a going concern. There is substantial doubt regarding our ability to continue as a going concern. Our continued existence is dependent upon our ability to obtain additional capital. As of December 31, 2024, our cash, cash equivalents and marketable securities were \$ 91. 9 million. Our management believes that such cash, cash equivalents and restricted cash will not be sufficient to fund our operating expenses and capital requirements for one year after the date that the financial statements included in this Annual Report are issued. We will require significant additional funding to advance any of our product candidates beyond the short term. Our ability to continue as a going concern will depend on our ability to obtain additional funding, as to which no assurances can be given. We continue to analyze various alternatives, including additional debt or equity financings or other arrangements.** We will need substantial additional funding. If we are unable to raise capital when needed, we would be forced to delay, reduce or eliminate our research and product development programs or future commercialization efforts. We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the clinical development of trem- cel

in acute myeloid leukemia (“AML and MDS and”), advance our VCAR33 programs through clinical development, initiate clinical development of trem- cel in combination or in sequence with VCAR33ALLO as a targeted therapeutic, which we refer to as the trem- cel VCAR33 Treatment System, and otherwise continue to advance our research programs in support of our pipeline. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of a collaborator. Further In addition, we expect to continue to incur significant additional costs associated with operating as a public company this year and in future years. Accordingly, we will need to obtain substantial additional funding in order to maintain our continuing operations. 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 product development programs or future commercialization efforts. As of December 31, 2023-2024, our cash, cash equivalents and marketable securities were \$ 137.91, 2.9 million. We expect that our existing cash, cash equivalents and marketable securities as of December 31, 2023-2024 will enable us to fund our operating expenses and capital expenditure requirements into the second half first quarter of 2025-2026. However, our operating plan may change as a result of factors currently unknown to us, and we may need to seek funding sooner than planned. Our future capital requirements will depend on many factors, including:

- the progress, results and costs of clinical trials for our product candidates;
- the costs of continuing to build our technology platform, including in- licensing additional genome engineering technologies for use in developing our product candidates;
- the costs of researching, developing, acquiring or in- licensing additional targeted therapies to use in combination or in sequence with trem- cel and other eHSC product candidates;
- the scope, progress, results and costs of discovery, preclinical development, formulation development and clinical trials for other product candidates;
- the costs of expanding our facilities to accommodate corporate, laboratory, and manufacturing needs, including commercial manufacturing;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property and proprietary rights and defending intellectual property- related claims in the United States and internationally;
- the costs, timing and outcome of regulatory review of any product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, distribution, coverage and reimbursement for any product candidates for which we receive regulatory approval;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the success of our collaborations, including ones we may establish, and of our license agreements;
- the achievement of milestones or occurrence of other developments that trigger payments under any collaboration agreements we enter;
- the extent to which we acquire or in- license product candidates, intellectual property and technologies;
- the extent to which we develop or in- license manufacturing and distribution technologies; and
- the costs of operating as a public company.

Conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, even if we successfully develop product candidates and those are approved, we may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for several years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Any additional fundraising efforts may divert our management from their day- to- day activities, which may adversely affect our ability to develop and commercialize product candidates. We cannot be certain that additional funding will be available on acceptable terms, or at all. We have no committed source of additional capital and, if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of product candidates or other research and development initiatives. Our license agreements and any future collaboration agreements may also be terminated if we are unable to meet the payment or other obligations under the agreements. We could be required to seek collaborators for product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves. If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidate, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline. Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, government or private party grants, debt financings, collaborations, strategic alliances and licensing arrangements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, including through the use of our at- the- market facility, our stockholders’ ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect rights of our common stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, declaring dividends and possibly other restrictions. If we raise funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or we may have to grant licenses on terms that may not be favorable to us or commit to providing us with future payment streams. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Market volatility may further adversely impact our ability to access capital as and when needed. We have a limited operating history, have not yet completed any clinical trials and

have no history of commercializing products, which may make it difficult to evaluate the success of our business to date and to assess our future viability. We are an early- stage company. We were founded in December 2015 and commenced operations in February 2016. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring and developing our platform and technology, identifying product candidates and undertaking studies. For example, VBP101, our Phase 1 / 2a multicenter, open- label, first- in- human study of trem- cel in patients with AML, and VBP301, our Phase 1 / 2, multicenter, open- label, first- in- human study of ~~VCAR33ALLO~~ **VCAR33** in patients with relapsed or refractory AML, are each in the early stages and our other programs are still in the preclinical or research stage. The risk of failure for these activities is high. We have not yet demonstrated an ability to successfully complete any clinical trials, including large- scale, pivotal clinical trials, obtain marketing approvals, manufacture a commercial- scale product or arrange for a third party to do so on our behalf or conduct sales and marketing activities necessary for successful commercialization. For example, while we have demonstrated that our in- house cGMP clinical manufacturing facility at our Cambridge, MA headquarters can successfully manufacture clinical supply of ~~VCAR33ALLO~~ **VCAR33**, we may fail to fully realize the cost- savings and efficiency gains that we expect, and we may be unsuccessful in making arrangements with third parties for commercial manufacturing. Consequently, any predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history. Our limited operating history may make it difficult to evaluate our technology and industry and predict our future performance. Our short history as an operating company makes any assessment of our future success or viability subject to significant uncertainty. We expect to encounter risks and difficulties frequently experienced by early stage companies in new and rapidly evolving fields. If we do not address these risks and difficulties successfully, our business could suffer. In addition, as a new business, we may encounter other unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition. We have never generated revenue from product sales and may never become profitable. Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborators, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, product candidates. We do not anticipate generating revenues from product sales for the next several years, if ever. Our ability to generate future revenue from product sales depends heavily on our, or our current or future collaborators', ability to successfully:

- **advance and complete clinical trials of our product candidates, including trem- cel and VCAR33;**
- initiate and complete clinical development of other product candidates;
- complete research and preclinical and clinical development of any other product candidates we may identify;
- seek and obtain regulatory and marketing approvals for any product candidates for which we complete clinical trials;
- launch and commercialize any product candidates for which we obtain regulatory and marketing approval by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualify for coverage and adequate reimbursement by government and third- party payors for any product candidates for which we obtain regulatory and marketing approval;
- develop, maintain and enhance a sustainable, scalable, reproducible and transferable manufacturing process for our product candidates;
- establish and maintain supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for any product candidates for which we obtain regulatory and marketing approval;
- obtain market acceptance of product candidates as viable treatment options;
- address competing technological and market developments;
- implement internal systems and infrastructure, as needed;
- negotiate favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such arrangements;
- maintain, protect, enforce, defend and expand our portfolio of intellectual property rights, including patents, trade secrets and know- how, in the United States and internationally;
- avoid and defend against third- party interference, infringement and other intellectual property claims in the United States and internationally; and
- attract, hire and retain qualified personnel.

Even if one or more of the product candidates we develop are approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the U. S. Food and Drug Administration (the “ FDA ”), the European Medicines Agency (the “ EMA ”) or other regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved product candidates, we may not become profitable and may need to obtain additional funding to continue operations. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. 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 also could cause stockholders to lose all or part of their investment in us. Our ability to utilize our net operating loss carryforwards and certain other tax attributes to offset taxable income or taxes may be limited. As of December 31, ~~2023~~ **2024**, we had gross federal net operating loss carryforwards of \$ ~~190,243.29~~ **2.9** million including \$ ~~188,242.30~~ **3.0** million that had an indefinite carryforward period and \$ 1.9 million that were subject to expiration at various dates through 2037. Furthermore, we have state and local net operating loss carryforwards of \$ ~~180,234.87~~ **8.7** million which will expire at various dates through ~~2042~~ **2044**. Portions of these net operating loss carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act (the “ Tax Act ”), as modified by the Coronavirus Aid, Relief, and Economic Security (the “ CARES Act ”) U. S. federal net operating losses incurred in taxable years beginning after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal net operating losses in taxable years beginning after December 31, 2020, may be limited. It is uncertain how various states will respond to the Tax Act and the CARES Act. For state income tax purposes, there may be periods during which the use of net operating loss carryforwards is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the “ Code ”), and corresponding provisions of

state law, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50 % change, by value, in its equity ownership over a three- year period, the corporation’s ability to use its pre- change net operating loss carryforwards and other pre- change tax attributes to offset its post- change income or taxes may be limited. The completion of our initial public offering, together with private placements and other transactions that have occurred since our inception, may trigger such an ownership change pursuant to Section 382 of the Code. We have not yet completed a Section 382 analysis, and therefore, there can be no assurances that our net operating losses are not already limited. We may experience ownership changes as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. If an ownership change occurs and our ability to use our net operating loss carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations. There is a full valuation allowance for net deferred tax assets, including net operating loss carryforwards.

**†. Risks Related to Discovery, Development, Manufacturing and Commercialization** eHSCs is an emerging technology containing risk and might never lead to commercially viable products. We are developing trem- cel and other eHSCs for transplant into the human body. Although there have been significant advances in the field of genome engineering in recent years, these technologies have rarely been applied to hematopoietic stem cells (“HSCs”), and our approach is new and largely unproven. The scientific evidence to support the feasibility of developing eHSCs is limited. Successful development of eHSCs by us will require solving a number of challenges, including:

- obtaining regulatory authorization from the FDA and other regulatory authorities, which have limited or no experience with regulating the development and commercialization of eHSCs, to proceed with clinical trials;
- identifying appropriate genetic targets for modification within HSCs;
- developing and deploying consistent and reliable processes for procuring cells from consenting third- party donors, isolating HSCs from such donor cells, inactivating genetic targets within such HSCs, storing and transporting the resulting eHSCs for therapeutic use and finally infusing these eHSCs into patients;
- utilizing these eHSC product candidates in combination or in sequence with targeted therapeutics, which may increase the risk of adverse side effects;
- avoiding potential complications of eHSC transplants, including failure to engraft, rejection by host or lack of functionality, any of which could result in serious side effects or death;
- educating medical personnel regarding the potential side effect profile of our product candidates, particularly those that may be unique to our eHSCs;
- understanding and addressing variability in the quality of a donor’s cells, which could ultimately affect our ability to manufacture product in a reliable and consistent manner;
- developing processes for the safe administration of eHSC products, including long- term follow- up and registries, for all patients who receive these product candidates;
- relying on third parties to find suitable healthy donors;
- obtaining regulatory approval from the FDA and other regulatory authorities;
- manufacturing product candidates to our specifications and in a timely manner to support our clinical trials and, if approved, commercialization;
- sourcing clinical and, if approved by applicable regulatory authorities, commercial supplies for the materials used to manufacture and process product candidates;
- developing a manufacturing process and distribution network that can provide a stable supply with a cost of goods that allows for an attractive return on investment; and
- establishing sales and marketing capabilities ahead of and after obtaining any regulatory approval to gain market acceptance, and obtaining coverage, adequate reimbursement and pricing by third- party payors and governmental healthcare programs.

We have concentrated our research efforts to date on preclinical work to bring trem- cel into clinical development for the treatment of AML, and our future success is highly dependent on the successful development of eHSCs, such as trem- cel, and the therapeutic applications of these cells. We may decide to alter or abandon our initial programs as new data become available and we gain experience in developing eHSCs. We cannot be sure that our programs will yield satisfactory products that are safe and effective, scalable or profitable in our initial indication or any other indication we pursue. Moreover, actual or perceived safety issues, including as a result of adverse developments in our eHSC programs or in genome engineering programs undertaken by third parties or of the adoption of novel approaches to treatment, may adversely influence the willingness of subjects to participate in our clinical trials, or, if one of our product candidates is approved by applicable regulatory authorities, of physicians to subscribe to the novel treatment mechanics or of patients to provide consent to receive a novel treatment despite its regulatory approval. The FDA or other applicable regulatory authorities may require specific post- market studies or additional information that communicates the benefits or risks of our products. New data may reveal new risks of our product candidates at any time prior to or after regulatory approval. We are substantially dependent on the success of our two most advanced product candidates, trem- cel and ~~VCAR33ALLO~~ **VCAR33**. If we are unable to complete development of, obtain approval for and commercialize trem- cel or ~~VCAR33ALLO~~ **VCAR33** in a timely manner, our business will be harmed. Our future success is dependent on our ability to timely advance and complete clinical trials, obtain marketing approval for and successfully commercialize our product candidates trem- cel and ~~VCAR33ALLO~~ **VCAR33**. We are investing significant efforts and financial resources in the research and development of these product candidates. We released updated clinical data from VBP101, our Phase 1 / 2a multicenter, open- label, first- in- human trial of trem- cel in combination with Mylotarg in patients with AML, most recently in December ~~2023~~ **2024** based on ~~eight~~ **twenty- five** patients, and we are only in the early stages of advancing ~~VCAR33ALLO~~ **VCAR33** through clinical development. Trem- cel and ~~VCAR33ALLO~~ **VCAR33** will each require additional clinical development, evaluation of clinical, preclinical and manufacturing activities, marketing approval from government regulators, substantial investment and significant marketing efforts before we can generate any revenues from product sales. We are not permitted to market or promote trem- cel, ~~VCAR33ALLO~~ **VCAR33** or any other product candidate, before we receive marketing approval from the FDA and comparable foreign regulatory authorities, and we may never receive such marketing approvals. The success of trem- cel and ~~VCAR33ALLO~~ **VCAR33** will depend on several factors, including the following:

- the acceptance of individual investigational review boards (“IRBs”) and scientific review committees at each clinical trial site as to the adequacy of the preclinical data package to support clinical development of trem- cel and their overall general agreement with the use of trem- cel in the intended patient population in the intended manner;
- the willingness of clinical investigators to place patients in the clinical trials, and the willingness of patients to enroll in a clinical trial studying a first- in- human cell therapy;
- the

successful and timely completion of our Phase 1 / 2a clinical trial of trem- cel, the development of our ~~VCAR33ALLO~~ **VCAR33** program; • the initiation and successful patient enrollment and completion of additional clinical trials of trem- cel and ~~VCAR33ALLO~~ **VCAR33** on a timely basis; • maintaining and establishing relationships with contract research organizations (“ CROs ”) and clinical sites for the clinical development of these programs both in the United States and internationally; • the frequency and severity of adverse events in the clinical trials; • the results of clinical trials conducted by third parties in hematopoietic cell transplant (“ HCT ”) if such trials result in changes to the standard of care for HCT or otherwise cause us to change our clinical trial protocols; • the efficacy, safety and tolerability profiles that are satisfactory to the FDA, the EMA or any comparable foreign regulatory authority for marketing approval; • the timely receipt of marketing approvals for our programs from applicable regulatory authorities; • the extent of any required post- marketing approval commitments to applicable regulatory authorities; • the maintenance of existing or the establishment of new supply arrangements with third- party suppliers and manufacturers for clinical development of our programs; • the maintenance of existing, or the establishment of new, scaled production arrangements with third- party manufacturers to obtain, or the ability of our in- house manufacturing facility to produce, finished products that are appropriate for commercial sale of our programs, if either is approved; • obtaining and maintaining patent protection, trade secret protection and regulatory exclusivity, both in the United States and internationally; • the protection of our rights in our intellectual property portfolio; • the successful launch of commercial sales following any marketing approval; • a continued acceptable safety profile following any marketing approval; • commercial acceptance by patients, the medical community and third- party payors; • our ability to obtain coverage and adequate reimbursement from third- party payors for our products and patients’ willingness to pay out- of- pocket in the absence of such coverage and adequate reimbursement; and • our ability to compete with other treatments. We do not have complete control over many of these factors, including certain aspects of clinical development and the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing, distribution and sales efforts of any future collaborator. If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize trem- cel and / or ~~VCAR33ALLO~~ **VCAR33**, which would materially harm our business. If we do not receive marketing approvals for trem- cel and ~~VCAR33ALLO~~ **VCAR33** we may not be able to continue our operations. We may not be successful in our efforts to identify, develop and commercialize additional product candidates. If these efforts are unsuccessful, we may never become a commercial stage company or generate any revenues. The success of our business depends primarily upon our ability to identify, develop and commercialize additional product candidates based on, or complementary with, our technology platform. Other than our clinical trials for trem- cel and ~~VCAR33ALLO~~ **VCAR33**, all of our other product development programs are still in the research or preclinical stage of development. Our research programs may fail to identify additional product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates, our potential product candidates may be shown to have harmful side effects in preclinical in vitro experiments or animal model studies, they may not show promising signals of efficacy in such experiments or studies or they may have other characteristics that may make the product candidates impractical to manufacture, unmarketable or unlikely to receive marketing approval. The historical failure rate for product candidates is high due to risks relating to safety, efficacy, clinical execution, changing standards of medical care and other unpredictable variables. In addition, although we believe our technology platform will position us to rapidly expand our portfolio of product candidates beyond our current product candidates, our ability to expand our portfolio may never materialize. If any of these events occur, we may be forced to abandon our research or development efforts for a program or programs, which would have a material adverse effect on our business, financial condition, results of operations and prospects. Research programs to identify new product candidates require substantial technical, financial and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful, which would be costly and time- consuming. If our product candidates, the delivery modes we rely on to administer them, and / or the conditioning, administration process or related procedures or treatments which may be used alongside our product candidates cause serious adverse events, undesirable side effects or unexpected characteristics, such events, side effects or characteristics could delay or prevent regulatory approval of the product candidates, limit their commercial potential or result in significant negative consequences following any potential marketing approval, even if these side effects or characteristics are unrelated to our product candidate. We have not yet completed any human clinical trials of our product candidates and it is impossible to predict when or if our product candidates will prove safe in humans. Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or comparable foreign regulatory authorities. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. There have been limited clinical trials of eHSCs and a limited number of clinical trials of certain of the technologies we are using to engineer eHSCs and chimeric antigen receptor (“ CAR ”)- T cells, including the CRISPR / Cas9 method we are using in our trem- cel program. In the genetic medicine field, there have been several significant adverse events from genetically engineered treatments in the past, including reported cases of leukemia and death. There have also been studies suggesting that genome engineering using the CRISPR- Cas9 method may increase the risk that the modified cells themselves become cancerous or otherwise dysfunctional. There can be no assurance that our eHSCs or CAR- T cells and the genome engineering techniques that we may employ in their development will not cause undesirable side effects, as improper modification of a patient’ s DNA could lead to lymphoma, leukemia or other cancers, or other aberrantly functioning cells. A significant risk in any genetically engineered product candidate is that “ off- target ” gene alterations may occur, which could cause serious adverse events, undesirable side effects or unexpected characteristics. Although we and others have demonstrated the ability to improve the specificity of gene alterations in a laboratory setting, we cannot be certain that off- target alterations will not occur in any of our planned or future clinical trials, and the lack of observed side effects in preclinical studies does not

guarantee that such side effects will not occur in human clinical trials. There is also the potential risk of delayed adverse events following exposure to genetically engineered cells due to the permanence of changes to DNA or due to other components of product candidates used to carry the genetic material. Further, because our genome engineering technology makes a permanent change, the treatment cannot be withdrawn, even after a side effect is observed. For example, our eHSCs are designed to permanently reconstitute the blood cells necessary for the survival of HCT patients, and we cannot be certain that these changes will not induce adverse reactions in patients or impair the functionality of the resulting blood cells. The eHSC manufacturing process generally, and the removal of surface targets such as CD33 specifically, could have temporary or permanent harmful effects. While we have discovered anonymous individuals in genome databases who lack CD33, we cannot be certain that these databases are accurate or complete or that the individuals who have contributed DNA to the database are healthy, as comprehensive health information is not included in the databases we have consulted. The removal of CD33 or other surface targets we remove from HSCs could have serious harmful effects, including the impairment of the ability of our eHSCs to migrate to patients' bone marrow, survive and reconstitute properly functioning blood cells. These side effects may not be evident for years after transplant. In addition to side effects and adverse events that may be caused by our eHSCs, HCT is itself a complicated and risky procedure. The conditioning, administration process or related procedures which may be used in HCT can cause adverse side effects and adverse events. An HCT patient is generally administered cytotoxic drugs to remove stem cells from the bone marrow to create sufficient space in the bone marrow for the modified stem cells to engraft and produce new cells. This procedure compromises the patient's immune system. In addition, the HSCs administered via transplant may fail to engraft in patients' bone marrow, or could be rejected by the patient, either of which could result in serious side effects, including death. If in the future we are unable to demonstrate that such adverse events were caused by the elements of the HCT process unrelated to our eHSCs, the FDA, the European Commission, the Competent Authorities of the Member States of the European Union, EMA or other regulatory authorities could order us to cease further development of, or deny approval of, our eHSCs for any or all target indications. Even if we are able to demonstrate that adverse events are not related to our product candidates, or are merely a feature of HCT generally, such occurrences could affect patient recruitment, the ability of enrolled patients to complete the clinical trial, or the commercial viability of any product candidates that obtain regulatory approval. Furthermore, in previous and ongoing clinical trials involving CAR- T or other cell- based therapies from other companies, patients experienced side effects such as neurotoxicity and cytokine release syndrome. There have been life threatening events related to severe neurotoxicity and cytokine release syndrome, requiring intense medical intervention such as intubation or pressor support, and in several cases, resulting in death. Severe neurotoxicity is a condition that is currently defined clinically by cerebral edema, confusion, drowsiness, speech impairment, tremors, seizures, or other central nervous system side effects, when such side effects are serious enough to lead to intensive care. In some cases, severe neurotoxicity was thought to be associated with the use of certain lymphodepletion regimens used prior to the administration of the CAR- T or other cell- based therapies. Cytokine release syndrome is a condition that is currently defined clinically by certain symptoms related to the release of cytokines, which can include fever, chills, low blood pressure, when such side effects are serious enough to lead to intensive care with mechanical ventilation or significant vasopressor support. The exact cause or causes of cytokine release syndrome and severe neurotoxicity in connection with treatment of CAR- T or other cell- based therapies is not fully understood at this time. In addition, patients have experienced other adverse events in these trials, such as a reduction in the number of blood cells (in the form of neutropenia, thrombocytopenia, anemia or other cytopenias), febrile neutropenia, chemical laboratory abnormalities (including elevated liver enzymes) and renal failure. The delivery modalities for the production of certain of our product candidates may also cause serious adverse events. For example, in order to manufacture ~~VCAR33ALLO~~ **VCAR33**, we employ viral vectors, including lentiviruses, which are relatively new approaches. In past clinical trials that were conducted by others with lentivirus vectors, several significant side effects were caused by gene therapy treatments, including reported cases of leukemia and death. Other potential side effects could include an immunologic reaction and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation. If the vectors we use demonstrate a similar side effect, or other adverse events, we may be required to halt or delay further clinical development of ~~VCAR33ALLO~~ **VCAR33** and potential product candidates. Furthermore, the FDA has stated that lentiviral vectors possess characteristics that may pose high risks of delayed adverse events. Undesirable side effects caused by ~~VCAR33ALLO~~ **VCAR33** or other cell- based targeted therapeutics we may develop could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of marketing approval by the FDA or other comparable foreign regulatory authorities. In some cases, side effects such as neurotoxicity or cytokine release syndrome have resulted in clinical holds of ongoing clinical trials and / or discontinuation of the development of the product candidate. Results of our studies could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Treatment- related side effects could also affect patient recruitment or the ability of enrolled patients to complete the trials or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff, as toxicities resulting from T cell- based immunotherapies are not normally encountered in the general patient population and by medical personnel. Medical personnel may need additional training regarding T cell- based immunotherapy product candidates to understand their side effects. Inadequate training in recognizing or failure to effectively manage the potential side effects of T cell- based immunotherapy product candidates could result in patient deaths. Any of these occurrences may harm our business, financial condition and prospects significantly. If any product candidates we develop are associated with serious adverse events, undesirable side effects or unexpected characteristics, we may need to abandon their development or limit development to certain uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk- benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations, and prospects. Many product candidates

that initially showed promise in early stage testing have later been found to cause side effects that prevented further clinical development of the product candidates. Additionally, if we successfully develop a product candidate and it receives marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy (“REMS”), to ensure that the benefits of treatment with such product candidate outweighs the risks for each potential patient, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients, a communication plan to health care practitioners, extensive patient monitoring or distribution systems and processes that are highly controlled, restrictive and more costly than what is typical for the industry. Furthermore, if we or others later identify undesirable side effects caused by a product candidate, several potentially significant negative consequences could result, including: • regulatory authorities may suspend or withdraw approvals of such product candidate; • regulatory authorities may require additional warnings on the label or limit the approved use of such product candidate; • we may be required to change the way the product is administered, or implement other changes to the labeling or handling of a product, if approved; • we may be required to conduct additional clinical trials; • we could be sued and held liable for harm caused to patients; and • our reputation may suffer. Any of these events could prevent us from achieving or maintaining market acceptance of product candidates and could have a material adverse effect on our business, financial condition, results of operations and prospects. We ~~have not successfully tested~~ **are in the early stages of testing some of** our product candidates in clinical trials and any favorable preclinical results are not predictive of results that may be observed in clinical trials. We ~~have not successfully tested~~ **are only in the early stages of testing some of** our product candidates in clinical trials, and there is a high failure rate for drugs and biologics proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later stage clinical trials even after achieving promising results in earlier stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development. Any such adverse events may cause us to delay, limit or terminate planned clinical trials, any of which would have a material adverse effect on our business, financial condition, results of operations and prospects. In addition, the results of preclinical studies may not be predictive of the results of later- stage preclinical studies or clinical trials, and preliminary clinical data may not be predictive of later clinical data or the results of later- stage clinical trials. To date, we have generated only limited preclinical study data and preliminary clinical trial results, and any such data or results do not ensure that later preclinical studies or clinical trials will produce similar outcomes. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates. Furthermore, the IND for the T cell therapy product candidate using the same CAR construct as ~~VCAR33ALLO~~ **VCAR33**, which we refer to as VCAR33AUTO, is currently held, and this clinical trial is currently sponsored, by NMDP. As such, NMDP is responsible for all aspects of this trial, including the design of the trial, the manufacture of study product, the enrollment, dosing and follow- up of patients, the recording of trial data and the analysis of results. We also did not control the preclinical development of this T cell therapy product candidate, which was conducted by the National Institutes of Health (“NIH”), and we do not have rights under the license agreement to certain intellectual property, such as know- how, employed by NMDP in manufacturing study product or conducting its clinical trial. We have received the right to cross reference NMDP’ s IND for this T cell therapy product candidate in any future IND application we may make with the FDA. In the event we cross- reference these trial results, we will be required to demonstrate that our ~~VCAR33ALLO~~ **VCAR33** is comparable to the T cell therapy studied in NMDP trial, which will require us to show that our manufacturing processes and construct release specifications are sufficiently comparable to those employed in NMDP trial. While we do not believe that we need to demonstrate comparability of our ~~VCAR33ALLO~~ **VCAR33** candidate since we might rely on initial clinical data from our ~~VCAR33ALLO~~ **VCAR33** program, if the FDA disagrees, we may have to demonstrate comparability. Regulatory authorities may require us to obtain and submit additional preclinical, manufacturing or clinical data before we may initiate further clinical trials and / or obtain any regulatory approvals. For example, we may be required to conduct additional preclinical toxicology studies, requalify manufacturing processes or conduct further clinical investigation of ~~VCAR33ALLO~~ **VCAR33** before advancing our ~~VCAR33ALLO~~ **VCAR33** program. We are also relying on NIH to have conducted its research and development efforts, and on NMDP to conduct its clinical trial, in accordance with applicable protocol, legal, regulatory and scientific standards, to accurately report the results of preclinical studies and clinical trials, and to correctly collect and interpret the data from these studies and trials. To the extent any of these has not occurred or does not occur, the expected time and costs of developing our ~~VCAR33ALLO~~ **VCAR33** program, as well as the trem- cel VCAR33 Treatment System, may be increased, which could adversely affect our business. Furthermore we do not control the timing of the ongoing NMDP trial or the release of information about the trial, including trial results, all of which negatively affect our ability to accurately estimate the timing of anticipated trial milestones. As a result, our estimates may prove to be inaccurate. Additionally, our ability to conduct clinical development of ~~VCAR33ALLO~~ **VCAR33** could be delayed or otherwise adversely affected. NMDP also may not publicize data from the trial in a manner that facilitates further clinical development by us, or at all. NMDP may elect to publicize this data at a time or in a manner other than we desire or may interpret data from these trials in a manner differently than we do, any of which could harm our business. Development of a product candidate such as trem- cel, which is intended for use in combination or in sequence with an already approved therapy, will present increased complexity and more or different challenges than development of a product candidate for use as a single agent. We expect that our product candidate trem- cel, and any other eHSC product candidates that we may develop, will be required to be used in combination or in sequence with existing or future therapies in order to demonstrate more anti- cancer efficacy than unmodified HSCs. In particular, our Phase 1 / 2a clinical trial evaluates trem- cel in combination with Mylotarg and we anticipate conducting future trials of trem- cel with ~~VCAR33ALLO~~ **VCAR33** as a Treatment System, and also

potentially with other targeted therapies. Developing product candidates for use in combination or sequence with other therapies will present challenges. For example, the FDA may require us to use more complex clinical trial designs to evaluate the contribution of each product and product candidate to any observed effects. Moreover, following product approval, the FDA may require that products used in conjunction with each other be cross-labeled, which would require consent from the sponsoring company. To the extent that we do not have rights to already approved products, this may require us to work with another company to satisfy such a requirement. For example, we do not have and do not currently plan to enter into a cross-labeling agreement with Pfizer with respect to Mylotarg, and therefore any such cross-labeling requirement from the FDA would require us to negotiate such an agreement with Pfizer. In addition, developments related to the already approved therapies may impact our clinical trials for the combination as well as our commercial prospects should we receive marketing approval. Such developments may include changes to the approved therapy's safety or efficacy profile, changes to the availability of the approved therapy, changes to the standard of care and a decision by the sponsoring company to withdraw the therapy from the market. For example, Mylotarg was voluntarily withdrawn from the market in 2010 after post-approval testing indicated increased risks of hepatic veno-occlusive disease, or blockage of veins in the liver. Mylotarg was re-approved in 2017 with a lower recommended dose and for use in a new patient population. Also, while we do not currently require a license from or agreement with Pfizer to permit us to conduct clinical trials or, if approved, to commercialize trem-cel with Mylotarg as a targeted therapeutic, we do not have and do not plan to enter into a supply or license agreement with Pfizer that would require Pfizer to produce Mylotarg, or permit us to otherwise produce Mylotarg, for these purposes. If Mylotarg undergoes subsequent labeling changes, or if Mylotarg is again removed from the market due to renewed concerns about its safety profile, or for other reasons, our clinical trial of trem-cel, and our prospects for commercializing trem-cel, might be materially adversely affected. Further, we believe trem-cel could unlock the potential of anti-CD33 therapies, such as ~~VCAR33ALLO~~ **VCAR33**, that are much more potent than Mylotarg and are not associated with severe myeloablative toxicities. While VBP101, our Phase 1 / 2a multicenter, open-label, first-in-human study of trem-cel in patients with AML, is not designed to evaluate the efficacy of the combination of trem-cel and Mylotarg, the clinical data for trem-cel in combination with Mylotarg may not reflect the potential efficacy of trem-cel in the long-term. For example, in February 2022, we announced that the first patient enrolled in VBP101 was moved to other therapies following administration of the third dose of Mylotarg due to detectable measurable residual disease, and subsequently relapsed, despite the patient maintaining neutrophil and platelet counts approximately five months after transplantation with trem-cel. Patient completion of our clinical trials could be impacted by the efficacy of Mylotarg or any other therapy administered in combination with our product candidates. Furthermore, we will not be able to market and sell trem-cel or any product candidate we develop in combination with an unapproved cancer therapy, such as VCAR33 or other cell-based targeted therapeutics, for a combination indication, if that unapproved therapy does not ultimately obtain marketing approval either alone or in combination with our product. To our knowledge, the FDA has not previously approved combined cell therapies, and we cannot be certain whether the FDA will apply existing guidance to cell therapies product candidates, such as the trem-cel VCAR33 Treatment System, or will otherwise apply existing guidance in novel ways. In addition, unapproved cancer therapies face the same risks described with respect to our product candidates currently in development and clinical trials, including the potential for serious adverse effects, delay in their clinical trials and lack of FDA approval. If the FDA, EMA or comparable foreign regulatory authorities do not approve these other drugs or revoke their approval of, or if safety, efficacy, quality, manufacturing or supply issues arise with, the drugs we choose to evaluate in combination with any product candidate we develop, we may be unable to obtain approval of or market such combination therapy. Any inability to develop targeted therapies for use with our product candidate, any failure to maintain or enter into new successful commercial relationships with respect to targeted therapies, or the expense of purchasing targeted therapies in the market, may delay our development timelines, increase our costs and jeopardize our ability to develop our current product candidates and any future product candidates as commercially viable therapies. If any of these occur, our business, financial condition, results of operations, stock price and prospects may be materially harmed. If we are unable to successfully develop our current programs into a comprehensive portfolio of product candidates, or experience significant delays in doing so, we may not realize the full commercial potential of our current and future product candidates. We are developing trem-cel so that it can be used in combination or in sequence with other product candidates that we in-license or develop ourselves, and we are focused on a product development strategy that includes leveraging the synergies among a comprehensive portfolio of our product candidates. For example, if the initial clinical trials of trem-cel and ~~VCAR33ALLO~~ **VCAR33** are each successful, we anticipate conducting clinical trials of the trem-cel VCAR33 Treatment System, for the treatment of myeloid malignancies such as AML. Our development of the trem-cel VCAR33 Treatment System will involve additional process development and could require additional regulatory submissions, such as an IND. Our success may depend, in part, on our ability to develop a complementary product portfolio with product candidates that will address a major limitation of existing therapies. Given our limited experience in developing product candidates that have received marketing approval, we may not be successful in developing some of our product candidates. The failure of one of our product candidates to obtain regulatory approval or market acceptance may affect our ability to expand our market opportunities for our other product candidates or programs. Although we may develop product candidates that ultimately obtain marketing approval, if we are unable to successfully develop our current programs into a comprehensive portfolio of product candidates, or experience significant delays in doing so, we may not realize the full commercial potential of our current and future product candidates. 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 focus on product candidates and research programs that we identify for specific indications among many potential options. 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 product candidates and research and development programs 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 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. Any such event could have a material adverse effect on our business, financial condition, results of operations and prospects. Even if a product candidate receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success. The commercial success of our product candidates, if approved, will depend upon their degree of market acceptance by physicians, patients, third-party payors and others in the medical community. Ethical, social and legal concerns about genetic medicines generally and genome engineering technologies specifically could result in additional regulations restricting or prohibiting the marketing of our product candidates. Even if any product candidate we develop receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors and others in the medical community. The degree of market acceptance of any product candidate we develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidate as demonstrated in clinical trials;
- the efficacy and safety of other products that are used in combination or in sequence with our product;
- the potential and perceived advantages of our product candidates compared to alternative treatments;
- the limitation to our targeted patient population and limitations or warnings contained in approved labeling by the FDA or other regulatory authorities;
- the ability to offer our products for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA, the EMA or other regulatory agencies authorities;
- public attitudes regarding genetic medicine generally and genome engineering technologies specifically;
- the willingness of the target patient population to try novel biologics and of physicians to prescribe these treatments, as well as their willingness to accept an intervention that involves the alteration of the patient's gene;
- product labeling or product insert requirements of the FDA, the EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments;
- the strength of marketing and distribution support;
- availability of third-party coverage and sufficiency of reimbursement; and
- the prevalence and severity of any side effects.

Even if a product candidate is approved, such product may not achieve an adequate level of acceptance, we may not generate significant product revenues, and we may not become profitable. If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market our product candidates, we may not be successful in commercializing those product candidates if and when they are approved. We do not have a sales or marketing infrastructure and have limited experience in the sale, marketing or distribution of pharmaceutical products. 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 or outsource these functions to third parties. In the future, we may choose to build a focused sales, marketing and commercial support 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 commercial capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force or reimbursement specialists 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 and other commercialization 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 commercialization personnel. Factors that may inhibit our efforts to commercialize our product candidates on our own include:

- our inability to recruit and retain adequate numbers of effective sales, marketing, reimbursement, customer service, medical affairs and other support personnel;
- the inability of sales personnel to obtain access to physicians or educate adequate numbers of physicians on the benefits of prescribing any future products;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement and other acceptance by payors;
- restricted or closed distribution channels that make it difficult to distribute our product candidates to segments of the patient population;
- 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 lines; and
- unforeseen costs and expenses associated with creating an independent commercialization organization.

If we enter into arrangements with third parties to perform sales, marketing, commercial support and distribution services, our product revenues or the profitability of these product revenues to us may be lower than if we were to market and sell products ourselves. In addition, we may not be successful in entering into arrangements with third parties to commercialize our product candidates or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish commercialization capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates. We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize product candidates, if approved. The development and commercialization of new drug and biologic products is highly competitive. Moreover, the genome engineering and oncology fields are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. We will face competition with respect to our product candidates that we develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies and other

public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we have product candidates and research programs. Some of these competitive products and therapies are based on scientific approaches that are similar to our approach, and others are based on entirely different approaches. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future that are approved to treat the same diseases for which we may obtain approval for our product candidates. This may include other types of therapies, such as small molecule, antibody and / or protein therapies. Many of our current or potential competitors, either alone or with their collaboration partners, may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and gene therapy industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These 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, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize product candidates that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than our product candidates or that would render our product candidates obsolete or non-competitive. Our competitors also may obtain FDA or other regulatory approval for their product candidates 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. Additionally, technologies developed by our competitors may render our product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates against competitors. In addition, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and / or scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for our product candidates, if approved. Adverse public perception of genetic medicines, and of genome engineering in particular, including as a result of other trials out of our control, such as the VCAR33AUTO trial currently sponsored by NMDP, may negatively impact regulatory approval of, and / or demand for, our potential products. Trem-cel, and future eHSCs and CAR-T or other cell-based targeted therapeutics we may develop, including product candidates that are evaluated in clinical trials out of our control, such as the VCAR33AUTO trial currently sponsored by NMDP, will be created by altering the human genome. The clinical and commercial success of our potential products will depend in part on public understanding and acceptance of the use of genome engineering for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that genome engineering is unsafe, unethical or immoral, and, consequently, our current or future product candidates may not gain the acceptance of the public or the medical community. In addition, developments related to already approved gene therapies, such as Casgevy, may impact public attitudes towards genome editing technology, including changes to the approved therapy's safety or efficacy profile, changes to the availability of the approved therapy, changes to the standard of care or a decision by the sponsoring company to withdraw the therapy from the market. Adverse public attitudes may adversely impact the ability to enroll clinical trials for our current or future product candidates. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available. In addition, genome engineering technology is subject to public debate and heightened regulatory scrutiny due to ethical concerns relating to the application of genome engineering technology to human embryos or the human germline. For example, in the United States, germline alteration for clinical application has been expressly prohibited since enactment of a December 2015 FDA ban on such activity. Prohibitions are also in place in the United Kingdom, across most of Europe, in China and many other countries around the world. In the United States, the NIH has announced that the agency would not fund any use of gene engineering technologies in human embryos, noting that there are multiple existing legislative and regulatory prohibitions against such work, including the Dickey-Wicker Amendment, which prohibits the use of appropriated funds for the creation of human embryos for research purposes or for research in which human embryos are destroyed. Although our product candidates do not involve technologies to alter human embryos or the human germline, public debate about the use of genome engineering technologies in human embryos and heightened regulatory scrutiny could prevent or delay the development of our product candidates. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development, commercialization and demand of our current or future product candidates. Adverse events in the preclinical studies or clinical trials for our current or future product candidates or those of our competitors or of academic researchers utilizing genome engineering technologies, even if not ultimately attributable to product candidates we may identify and develop, and the accompanying publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of potential product candidates we may identify and develop, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. Use of genome engineering technology by a third party or government to develop biological agents or products that threaten U. S. national security could similarly result in such negative impacts to us. Due to the novel nature of our eHSCs, the small patient population we are addressing and the potential for any of our product candidates to offer benefits in a single administration or limited number of administrations, we face additional uncertainty related to pricing, coverage and reimbursement for these product candidates. The pricing and reimbursement of our product candidates, if approved, must be adequate to support the necessary commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully

market and sell any such product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to a product candidate (e. g., for administration of our product candidates to patients) is also important. Inadequate reimbursement for such services may lead to physician and payor resistance and adversely affect our ability to market or sell any product candidate we develop. We are initially developing product candidates targeting rare diseases with small patient populations. For products that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such products must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate with a smaller patient population that accounts for the smaller potential market size. Even if we obtain coverage for a given product by a third- party payor, the resulting reimbursement payment rates may not be adequate. We are also initially developing products that are designed to be used in a single administration. We expect the cost of a single administration of genetic treatments, such as those we are seeking to develop, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by governmental healthcare programs such as Medicare and Medicaid, private health insurers and other third- party payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of any such product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of any such product candidates will be paid by governmental healthcare programs, private health plans and other third- party payors. Payors may not be willing to pay high prices for a single administration. Coverage and reimbursement by a third- party payor and physician utilization may depend upon several 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. There is significant uncertainty related to third- party coverage and reimbursement of eHSCs. For example, effective for cost reporting periods beginning on or after October 1, 2020, under the Medicare Hospital Inpatient Prospective Payment Systems (“ IPPS ”), Medicare payment to the hospital for hematopoietic stem cell acquisition, including the preparation and processing of stem cells derived from peripheral blood, will be made on a reasonable cost basis. We believe that this new rule may also apply to eHSC products. Alternatively, we may apply for Medicare’ s New Technology Add- on Payment (“ NTAP ”) designation for our eHSC product candidates, which, if approved, may allow for temporary reimbursement for new cell therapies above the standard Medicare Severity Diagnosis- Related Group payment amount under IPPS. NTAP will only be available for our product candidates, if approved, if we submit a timely and complete application and the Centers for Medicare & Medicaid Services (“ CMS ”) determines that our product candidates meet the eligibility requirements of NTAP, including, among other criteria, demonstrating a substantial clinical improvement relative to services or technologies previously available. We also believe that, for patients covered by commercial insurance, reimbursement will be based on a case rate methodology with possible provisions for separate payments for new therapies, such as eHSC. However, we cannot be certain that our eHSCs would qualify for these carveouts or other reimbursement avenues for new therapies. We also may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize a product candidate. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment. Further, even if favorable coverage and reimbursement status is attained for one or more product candidates for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell any product candidates will be harmed. We may need to develop new reimbursement models to realize adequate value for our product candidates. Payors may not be able or willing to adopt such new models, and patients may be unable to afford that portion of the cost that such models may require them to bear. If we determine such new models are necessary but we are unsuccessful in developing them, or if such models are not adopted by payors, our business, financial condition, results of operations and prospects could be adversely affected. Additionally, we or our collaborators may develop companion diagnostic tests for use with our product candidates. Companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical products, will apply to companion diagnostics. Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost- containment initiatives in Europe and other countries has and will continue to put pressure on the pricing and usage of our products. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our products. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits. Our inability to promptly obtain coverage and profitable payment rates from both government- funded and private payors for any approved products we may 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. The market for our product candidates, if approved, may be limited to those patients who are ineligible for or have failed, or are at risk of failing, prior treatments and who are able to tolerate the side effects of co- administered or sequentially administered targeted therapies, and our projections regarding the size of the addressable market may be incorrect. Cancer therapies are sometimes characterized as first line, second line or third line, and the FDA often approves new therapies initially only for last line use. When blood cancers are detected, they are treated with first line of therapy with the intention of curing the cancer. This generally consists of chemotherapy, radiation, antibody drugs, tumor- targeted small molecules or a combination of these. In addition, for myeloid malignancies, HCT is frequently added to the first line therapy after the combination chemotherapy is given. If the patient’ s cancer relapses, then they are given a second line or third line therapy, which can consist of more chemotherapy, radiation, antibody drugs, tumor- targeted small

molecules or a combination of these, or HCT. Generally, the higher the line of therapy, the lower the chance of a cure. If a patient relapses after HCT, the goal of the therapy in the treatment of AML is to control the growth of the tumor and extend the life of the patient, as a cure is unlikely to happen. We are initially developing trem- cel for use in patients receiving HCT who have been determined to be at high- risk for relapse of AML in the anticipation that trem- cel would enhance the utility and broaden the applicability of therapies subsequently deployed. ~~VCAR33ALLO~~---- **VCAR33** or any other targeted therapeutic we may develop is not guaranteed approval as an earlier line therapy or in settings other than bridge to transplant. In addition, we may have to conduct additional large randomized clinical trials prior to or post gaining approval for use trem- cel in patients who have not experienced relapse and / or in combination with an earlier line of therapy or of ~~VCAR33ALLO~~---- **VCAR33** as or in combination with a different line of treatment. Our projections of both the number of people who have the cancers we are targeting, as well as the size of the patient population subset who are in a position to undergo HCT, who are likely to relapse and who have the potential to benefit from treatment with eHSCs, or who are in a position to benefit from a targeted therapeutic, such as ~~VCAR33ALLO~~---- **VCAR33**, are based on our estimates and data provided to us by third parties. These estimates have been derived from a variety of sources, including scientific literature, surveys of clinics, NMDP, research facilities, patient foundations or market research and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these cancers. The number of patients may turn out to be fewer than expected. Additionally, the potentially addressable patient population for our product candidates may be limited, or may not be amenable to treatment with our product candidates. The addressable patient population will ultimately depend upon, among other things, the diagnosis criteria included in the final label, the availability of alternative treatments and the safety, convenience, cost and efficacy of our product candidates relative to such alternative treatments, acceptance by the medical community and patient access, drug pricing and reimbursement. Even if we obtain significant market share for our product candidates, because the potential target populations are small, we may never achieve significant revenue without obtaining regulatory approval for additional indications or in connection with earlier lines of therapy. If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates. We face an inherent risk of product liability exposure related to the testing in human clinical trials of our product candidates and will face an even greater risk if we commercially sell any products that we may develop. For example, we may be sued if our product candidates cause, or are perceived to cause, injury or are found to be otherwise unsuitable during clinical trials, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in: • the inability to commercialize any products that we may develop; • decreased demand for our product candidates or products that we may develop; • injury to our reputation and significant negative media attention; • withdrawal of clinical trial participants; • significant time and costs to defend the related litigation; • substantial monetary awards to trial participants or patients; and • loss of revenue. Insurance coverage is also increasingly expensive and we may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. Cell and genetic medicines are novel, and our product candidates are complex and difficult to manufacture. We could experience delays in satisfying regulatory authorities or production problems that result in delays in our development or commercialization programs, limit the supply of our product candidates, or otherwise harm our business. Our product candidates require processing steps that are more complex than those required for most chemical and other biological pharmaceuticals. Moreover, unlike chemical and other biological pharmaceuticals, the physical and chemical properties of a gene- engineered cell therapy, such as an eHSC or CAR- T or other cell- based targeted therapeutics we may develop, generally cannot be fully characterized. As a result, assays of the finished product candidate may not be sufficient to ensure that the product candidate will perform in the intended manner. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims, insufficient inventory or potentially delay progression of our potential IND filings or clinical trials. If we successfully develop product candidates, we may encounter problems achieving adequate quantities and quality of clinical- grade materials that meet FDA, EMA or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs. In addition, our product candidates will require complicated delivery modalities, such as electroporation, which will introduce additional complexities in the manufacturing process. Any of the foregoing factors could limit our ability to replicate the vein- to- vein time achieved in our preclinical manufacturing of trem- cel in a clinical or, if approved, commercial setting. Our product candidates consist, and any other eHSC or CAR- T or other cell- based targeted therapeutics we may develop will consist, of genetically engineered human cells, and the process of manufacturing such product candidates is complex, concentrated with a limited number of suppliers, highly regulated and subject to numerous risks. Manufacturing such product candidates involves harvesting cells from a donor or from the patient, altering the cells ex vivo using genome engineering technology, cryopreservation, storage and eventually shipment and infusing the cell product into the patient' s body. Our manufacturing process will be susceptible to product loss or failure, or product variation that may negatively impact patient outcomes, due to logistical issues associated with the collection of starting material from the donor, shipping such material to the manufacturing site, shipping the final product back to the clinical trial recipient, preparing the product for administration, infusing the patient with the product, manufacturing issues or different product characteristics resulting from the differences in donor starting materials, variations between reagent lots, interruptions in the manufacturing process, contamination, equipment or reagent failure, improper installation or operation of equipment, vendor or operator error, inconsistency in cell growth and variability in product characteristics. Our manufacturing process, like that of a number of other cell therapy companies, is also characterized by limited numbers of suppliers, and in some cases sole source suppliers, with the manufacturing capabilities and know- how to create or source the materials, such as donor

marrow cells and electroporation machines, used in our cell manufacturing. While we pursue multiple sources for the critical components of our manufacturing process, we may not be successful in securing these additional sources at all or on a timely basis. Even minor deviations from normal manufacturing processes could result in reduced production yields, product defects and other supply disruptions. If microbial, viral or other contaminations are discovered in our product candidates or in any of the manufacturing facilities in which products or other materials are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. In addition, because trem- cel and ~~VCAR33ALLO~~ **VCAR33** are manufactured for each particular patient, we will be required to maintain a chain of identity with respect to materials as they move from the donor or patient to the manufacturing facility, through the manufacturing process and back to the clinical trial recipient. Maintaining a chain of identity is difficult and complex, and failure to do so could result in adverse patient outcomes, loss of product or regulatory action, including withdrawal of an approved product from the market. Any failure in the foregoing processes could render a batch of product unusable, could affect the regulatory approval of such product candidate, could cause us to incur fines or penalties or could harm our reputation and that of our product candidates. We may make changes to our manufacturing process, and change the sites of manufacture, including with respect to our in- house manufacturing capabilities, for various reasons, such as to control costs, achieve scale, decrease processing time, increase manufacturing success rate or for other reasons. Changes to our process made during the course of clinical development could require us to show the comparability of the product used in earlier clinical phases or at earlier portions of a trial to the product used in later clinical phases or later portions of the trial. Other changes to our manufacturing process made before or after commercialization could require us to show the comparability of the resulting product to the product candidate used in the clinical trials using earlier processes. Such showings could require us to collect additional nonclinical or clinical data from any modified process prior to obtaining marketing approval for the product candidate produced with such modified process. If such data are not ultimately comparable to that seen in the earlier trials or earlier in the same trial in terms of safety or efficacy, we may be required to make further changes to our process and / or undertake additional clinical testing, either of which could significantly delay the clinical development or commercialization of the associated product candidate, which would materially adversely affect our business, financial condition, results of operations and growth prospects. In addition, the FDA, the EMA and other regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay clinical trials or product launches, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects. We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to manage our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements. Given the nature of biologics manufacturing, there is a risk of contamination during manufacturing. Any contamination could materially harm our ability to produce product candidates on schedule and could harm our results of operations and cause reputational damage. Some of the raw materials that we anticipate will be required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of trem- cel could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially harm our development timelines and our business, financial condition, results of operations and prospects. Also, due to the short time between the collection of donor HSCs, the manufacturing of trem- cel and the shipment to a transplant center for use in HCT, there are limited opportunities for sterility testing and we anticipate that final testing may occur just before or after the administration trem- cel. Any delays in testing may delay administration of trem- cel and any administration prior to testing may result in positive bacterial tests and obligations to notify health authorities. Any problems in our manufacturing process, including at either our in- house manufacturing facility or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in internal or third- party manufacturing process or facilities, including our own facility that we are building, also could restrict our ability to ensure sufficient clinical material for any clinical trials we may be conducting or are planning to conduct and meet market demand for any product candidates we develop and commercialize. The process for treating cancer patients using T cell therapy or other cell- based targeted therapies is subject to human and systemic risks. The “ vein- to- vein ” cycle for treating cancer patients using T cell therapy or other cell- based targeted therapies typically takes approximately four to six weeks and involves a large number of steps and human participants. First, the patient’ s lymphocytes are isolated by apheresis at the clinical site and shipped to the manufacturing site. Under cGMP conditions at the manufacturing site, the patient’ s lymphocytes are thawed and washed and then enriched for CD33- positive T cells using specialized reagents. After overnight culture and T cell activation, the T cells are transduced using lentiviral vector transduction technology to introduce the CAR genetic construct into the enriched T cell population. At the completion of T cell transduction, the T cells are harvested, formulated into the final drug product and then cryopreserved for delivery to patients. Similar procedures may be used for other cell- based targeted therapies, such as a CAR natural killer cell therapy. In the United States, samples of the final product are subjected to several release tests which must fulfill specified criteria for the drug product to be released for infusion. These include sterility, identity, purity, potency and other tests. We are subject to stringent regulatory and quality standards for the T cell therapy treatment process. We cannot offer assurances that our quality control and assurance efforts will be successful or that the risk of human or systemic errors in these processes can be eliminated. Prior treatments can alter the cancer and negatively impact chances for achieving clinical activity with our CAR- T or other cell- based targeted therapies. Patients with hematological cancers typically receive

highly toxic chemotherapy as their initial treatments that can impact the viability of the T cells collected from the patient and may contribute to highly variable responses to CAR- T or other cell- based targeted therapies. In certain instances, we may use the allogeneic derived T cell fraction from the leukapheresis of the HLA- matched normal healthy donors as the starting material. Like the patient derived T cells, these donor- derived T cells may also display variability that will impact responses to ~~VCAR33ALLO~~---- **VCAR33** or other cell- based targeted therapeutics we may develop. Patients could also have received prior therapies that target the same molecule on the cancer cells as cell- based targeted therapeutics we may develop and thereby these patients may have cancer cells with low or no expression of the target. As a result, ~~VCAR33ALLO~~---- **VCAR33** or any other cell- based targeted therapeutics we may develop may not recognize the cancer cell and may fail to achieve clinical activity. For example, AML patients could have received a BCMA- targeting antibody drug conjugate BCMA- ADC like GSK2857916, BCMA targeting T cell engagers like AMG- 420 (Amgen) and CC- 93269 (Bristol- Myers Squibb), or similar products or product candidates prior to receiving VCAR33 or any other cell- based targeted therapeutics we may develop. If any product candidates we develop do not achieve a sufficient level of clinical activity, we may discontinue the development of that product candidate, which could have an adverse effect on the value of our common stock. We and any third- party manufacturers and any third- party collaborators may be unable to successfully scale- up manufacturing of our product candidates in sufficient quality and quantity, which would delay or prevent us from developing such product candidates and commercializing approved products, if any. Although we have demonstrated our internal cGMP manufacturing capabilities to produce clinical supply of ~~VCAR33ALLO~~---- **VCAR33**, in order to conduct clinical trials of our product candidates, we may need to work with third- party manufacturers to manufacture them in sufficient quantities if we are not able to produce sufficient quantities on our own. Our use of third- parties and multiple facilities require technology transfer of our processes. Technology transfer carries risk and could impact our spend, timelines and clinical supply. We, or our manufacturing partners or our third- party collaborators, may be unable to successfully increase the manufacturing capacity of our product candidates in a timely or cost- effective manner, or at all. We expect that each lot of trem- cel and ~~VCAR33ALLO~~---- **VCAR33** will need to be manufactured for a specific individual patient, and each lot will need to be individually tested and released for that patient. As a result, we may experience limited production capacity and be unable to meet the need of all patients who could benefit from treatment, if approved. In addition, quality issues may arise during scale- up activities. If we or our manufacturing partners or collaborators are unable to successfully scale up the manufacture of our current or future product candidates in sufficient quality and quantity, the development, testing and clinical trials of that product candidate may be delayed or infeasible, and marketing approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business. We have not yet developed a validated methodology for freezing and thawing large quantities of eHSCs or of VCAR33, which we believe will be required for the storage and distribution of our product candidates, and we may face additional logistical challenges in the distribution of our product candidates. We have not demonstrated that eHSCs or VCAR33, when manufactured for late stage clinical studies or at a commercial scale, can be frozen and thawed without damage in a cost- efficient manner and without degradation. We may encounter difficulties not only in developing freezing and thawing methodologies, but also in obtaining the necessary regulatory approvals for using such methodologies in treatment. If we cannot adequately demonstrate similarity of our frozen product to the unfrozen form to the satisfaction of the FDA, we could face substantial delays in our regulatory approvals. If we are unable to freeze eHSCs or VCAR33 or other cell- based targeted therapeutics we may develop for shipping purposes, our ability to promote adoption and standardization of our products, as well as achieve economies of scale by centralizing production facilities, will be limited. Even if we are able to successfully freeze and thaw eHSCs or VCAR33 at commercial scale, we will still need to develop a cost- effective and reliable distribution and logistics network, which we may be unable to accomplish. We may face logistical challenges in shipping and tracking, which could increase our costs or result in delayed distribution. For these and other reasons, we may not be able to manufacture and distribute eHSCs, VCAR33 or other cell- based targeted therapeutics we may develop at commercial scale or in a cost- effective manner. If we or any contract manufacturers and suppliers that we engage 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 any contract manufacturers and suppliers we engage are subject to numerous federal, state and local environmental, health and safety laws, regulations and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. Our operations also produce hazardous waste. 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. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at our current or past facilities and at third- party facilities. We also could incur significant costs associated with civil or criminal fines and penalties. Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our product development and research efforts. In addition, we cannot eliminate the risk of accidental injury or contamination from these materials or wastes. 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 carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended, which could have a material adverse effect on our business, financial condition, results

of operations and prospects. In addition, we may incur substantial costs to comply with current or future environmental, health and safety laws, regulations and permitting requirements. For example, our products are considered to contain genetically modified organisms or cells, which are regulated in different ways depending upon the country in which preclinical research or clinical trials are conducted. These current or future laws, regulations and permitting requirements may impair our research, development or production efforts. Failure to comply with these laws, regulations and permitting requirements also may result in substantial fines, penalties or other sanctions or business disruption, which could have a material adverse effect on our business, financial condition, results of operations and prospects. Any third- party contract manufacturers and suppliers we engage will also be subject to these and other environmental, health and safety laws and regulations. Liabilities they incur pursuant to these laws and regulations could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations and prospects. Success in preclinical studies or clinical trials may not be indicative of results in future clinical trials, particularly for our clinical trials that involve only a small number of patients. Results from preclinical studies are not necessarily predictive of future clinical trial results, and interim results of a clinical trial are not necessarily indicative of final results. Our clinical trials may involve a small number of patients, which makes it difficult to predict whether early results from these trials will be indicative of the final results of the trials or be replicated in future trials. For example, we are actively recruiting for VBP101, our Phase 1 / 2a multicenter, open- label, first- in- human trial of trem- cel in combination with Mylotarg in patients with AML, and we **most recently** released ~~initial~~ clinical data in December ~~2022~~ **2024** and February 2023 based on ~~two~~ **twenty- five** patients **treated with trem- cel in the VBP101 study**. Although we believe ~~the initial~~ **this interim** clinical data could provide important validating evidence of the potential of trem- cel and our broader eHSC approach, the final results of this trial may not be consistent with our interim results. For that reason, we do not know whether these candidates will be effective for the intended indications or safe in humans. Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. This failure to establish sufficient efficacy and safety could cause us to abandon clinical development of our product candidates. Risks Related to Regulatory Review If clinical trials of any of our product candidates fail to demonstrate safety and efficacy to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of such product candidates. Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates. We and our collaborators, if any, may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize any product candidates, including:

- delays in reaching a consensus with regulators on trial design;
- regulators, IRBs, independent ethics committees or scientific review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- delays in reaching or failing to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective CROs, and clinical trial sites;
- clinical trials of product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development or research programs;
- difficulty in designing well- controlled clinical trials due to ethical considerations which may render it inappropriate to conduct a trial with a control arm that can be effectively compared to a treatment arm;
- difficulty in designing clinical trials and selecting endpoints for diseases that have not been well- studied and for which the natural history and course of the disease is poorly understood;
- the number of patients required for clinical trials may be larger than we anticipate; enrollment of suitable participants in these clinical trials, which may be particularly challenging for some of the rare diseases we are targeting in our most advanced programs, may be delayed or slower than we anticipate; or patients may drop out of these clinical trials at a higher rate than we anticipate;
- our third- party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators, IRBs or independent ethics committees may require that we or our investigators suspend or terminate clinical research or clinical trials for various reasons, including noncompliance with regulatory requirements, a finding of undesirable side effects or other unexpected characteristics, or that the participants are being exposed to unacceptable health risks or after an inspection of our clinical trial operations or trial sites;
- the cost of clinical trials may be greater than we anticipate;
- the supply or quality of product candidates or other materials necessary to conduct clinical trials may be insufficient or inadequate, including as a result of delays in the testing, validation, manufacturing and delivery of product candidates to the clinical sites by us or by third parties with whom we have contracted to perform certain of those functions;
- delays in having patients complete participation in a trial or return for post- treatment follow- up;
- clinical trial sites dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with product candidates that are viewed to outweigh their potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols; and
- disruption in the supply or availability of Mylotarg or any future targeted therapeutics we use with our eHSCs.

If we or our collaborators are required to conduct additional clinical trials or other testing of product candidates beyond those that we currently contemplate, if we or our collaborators are unable to successfully complete clinical trials or other testing of product candidates, or if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns,

we or our collaborators may: • be delayed in obtaining marketing approval for any such product candidates or not obtain marketing approval at all; • 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 or suspend their approval of the product or impose restrictions on its distribution in the form of a REMS or through modification to an existing REMS; • be sued; or • experience damage to our reputation. Product development costs will also increase if we or our collaborators experience delays in clinical trials or other testing or in obtaining marketing approvals. We do not know whether any clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize product candidates, could allow our competitors to bring products to market before we do and could impair our ability to successfully commercialize product candidates, any of which may harm our business, financial condition, results of operations and prospects. Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize trem- cel, VCAR33, the trem- cel VCAR33 Treatment System or any other product candidate we may develop in the United States or any other jurisdiction, and any such approval may be for a more narrow indication than we seek. We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non- approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials and the review process. Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or a REMS. These regulatory authorities may require labeling that includes precautions or contra- indications with respect to conditions of use, or they may grant approval subject to the performance of costly post- marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and materially adversely affect our business, financial condition, results of operations and prospects. Marketing approval by the FDA in the United States, if obtained, does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product candidate testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional preclinical studies or clinical trials which could be costly and time- consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our product candidates in those countries. The foreign regulatory approval process involves all of the risks associated with FDA approval. 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, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of our product candidates will be unrealized. Genome engineering technology is subject to a number of challenges and risks. Because genome engineering technology is novel and the regulatory landscape that will govern our product candidates is uncertain and may change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for our product candidates. Because our product candidates and technology platform involve genome engineering, we are subject to many of the challenges and risks that other genetically engineered biologics and gene therapies face, including: • regulatory requirements or guidance regarding the requirements governing genome engineering products have changed and may continue to change in the future; • to date, only a limited number of products that involve genome engineering have been approved globally; • improper modulation of a gene sequence, including unintended alterations or insertion of a sequence into certain locations in a patient' s chromosomes, could lead to cancer, other aberrantly functioning cells or other diseases, as well as death; • transient expression of the Cas9 protein could lead to patients having an immunological reaction towards those cells, which could be severe or life- threatening; • corrective expression of a missing protein, or deletion of an existing protein, in patients' cells could result in the protein or cell being recognized as foreign, and lead to a sustained immunological reaction against the expressed protein or expressing cells, which could be severe or life- threatening; • regulatory agencies may require extended follow- up observation periods of patients who receive treatment using genome engineering products including, for example, the FDA' s recommended 15- year follow- up observation period for these patients, and we will need to adopt such observation periods for our product candidates if required by the relevant regulatory agency, which could vary by country or region; and • the field of genome engineering is subject to a number of intellectual property disputes. The regulatory requirements that will govern our novel genetically engineered product candidates are not entirely clear and may change. Moreover, serious adverse events or developments in clinical trials of gene therapy product candidates conducted by others may cause the FDA or other regulatory bodies to initiate a clinical hold on our clinical trials or otherwise change the requirements for approval of any of our product candidates. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation. The same applies in the European Union. Adverse developments in post- marketing experience or in clinical trials conducted by others of gene therapy products, cell therapy products or products developed through the

application of a genome engineering technology may cause the FDA, the EMA and other regulatory bodies to revise the requirements for development or approval of our product candidates or limit the use of products utilizing genome engineering technologies, either of which could materially harm our business. In addition, the clinical trial requirements of the FDA, the EMA and other regulatory authorities 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 trem- cel, can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing genome engineering technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays or other impediments to our product candidate development, research programs or the commercialization of resulting products. The regulatory review committees and advisory groups described above and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates, or lead to significant post- approval limitations or restrictions. Currently, OTAT requires a 15- year follow- up for each patient who receives a genetically engineered cell or gene therapy. This applies to all patients treated in trials during clinical development prior to approval. Following approval, such prolonged follow- up could continue to be required. As we advance our product candidates and research programs, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. Because we are developing product candidates using new technologies, as well as potential mechanisms of action for which there are few precedents, there is increased risk that the FDA, the EMA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze. The FDA, EMA and other regulatory authorities typically assess the safety and efficacy of a product with sufficient data to justify marketing authorization. We expect that trem- cel and any other eHSC product candidates we develop will not, by themselves, provide any anti- tumor activity in patients that relapse after HCT, and that our eHSCs could be effective after patients relapse only when administered in combination or sequence with other therapies. There are few precedents for product candidates with this potential mechanism of action. Furthermore, we are employing genome engineering technologies in the creation of our eHSCs that have not yet been clinically validated. During the regulatory review process, we will need to identify success criteria and endpoints such that the FDA, the EMA or other regulatory authorities will be able to determine the clinical efficacy and safety profile of our product candidates. As we are initially seeking to identify and develop product candidates to treat diseases using novel methods of action and new technologies, there is heightened risk that the FDA, the EMA or other regulatory authorities may not consider the clinical trial endpoints that we propose to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre- specified endpoints to a degree of statistical significance. Further, even if we do achieve the pre- specified criteria, we may produce results that are unpredictable or inconsistent with the results of the non- primary endpoints or other relevant data. The FDA also weighs the benefits of a product against its risks, and the FDA may view the efficacy results in the context of safety as not being supportive of regulatory approval. Other regulatory authorities in the European Union and other countries may make similar comments with respect to these endpoints and data. Our product candidates are based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. Interim “ top- line ” and preliminary results from our clinical trials that we may announce or publish from time to time 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. Investors and analysts may have difficulty analyzing our interim and preliminary results or may not consider them to be meaningful. From time to time, we may publish interim top- line or preliminary results from our preclinical studies and clinical trials, which are based on a preliminary analysis of then- available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. Interim results from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or top- line results 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. For example, we released clinical data from VBP101, our Phase 1 / 2a multicenter, open- label, first- in- human trial of trem- cel in combination with Mylotarg in patients with AML demonstrating neutrophil engraftment and platelet recovery, but these initial engraftment and platelet recovery results may not ultimately lead to efficacy of trem- cel. As a result, interim and preliminary data should be viewed with caution until the final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly. 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 investors or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product, product candidate or our business. The information we choose to publicly disclose may also be difficult for investors and analysts to analyze and they may not consider the data to be meaningful. If the interim, top- line or preliminary data that we report differ from actual results, or if others, including regulatory authorities, investors or

analysts, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition. If we experience significant delays or difficulties in the enrollment of patients in clinical trials, including with respect to completing a complex donor identification and screening process, the cost of developing product candidates could increase and our receipt of necessary regulatory approvals could be delayed or prevented. Patient enrollment is a significant factor in the timing of clinical trials. The timing of our clinical trials depends, in part, on the speed at which we can recruit patients to participate in our trials. We or our collaborators may not be able to advance clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA, the EMA or other analogous regulatory authorities outside the United States, or as needed to provide appropriate statistical power for a given trial. Patients may be unwilling to participate in our clinical trials because of negative publicity from adverse events related to the biotechnology, gene therapy or genome engineering fields, competitive clinical trials for similar patient populations, clinical trials in competing products or for other reasons. As a result, the timeline for recruiting patients, conducting trials and obtaining regulatory approval of product candidates **could** be delayed. Patient enrollment is also affected by other factors, including:

- severity of the disease under investigation;
- size of the patient population and process for identifying patients;
- completion of a complex donor identification and screening process and willingness of a donor to participate;
- prospective donors being registered on the requisite donor registry in order to participate in the trial;
- design of the trial protocol;
- availability and efficacy of approved medications for the disease under investigation;
- availability of genetic testing for potential patients;
- ability to obtain and maintain patient informed consent;
- risk that enrolled patients will drop out before completion of the trial, including due to side effects or characteristics that are unrelated to our product candidate;
- eligibility and exclusion criteria for the trial in question;
- perceived risks and benefits of the product candidate under trial;
- perceived risks and benefits of genome engineering as a treatment approach;
- perceived risks and benefits of the targeted therapeutics that may be administered in combination or in sequence with trem- cel or our other eHSC product candidates;
- efforts to facilitate timely enrollment in clinical trials;
- potential disruptions caused by epidemics or pandemics, including difficulties in initiating clinical sites, enrolling and retaining participants, diversion of healthcare resources away from clinical trials, travel or quarantine policies that may be implemented, and other factors;
- patient referral practices of physicians;
- ability to monitor patients adequately during and after treatment;
- proximity and availability of clinical trial sites for prospective patients, especially for those conditions which have small patient pools;
- the requirement for HCT to be performed in centers that specialize in this procedure; and
- changes to diagnostic technologies, methodologies or criteria used to identify HCT patients at high risk for relapse.

Significant enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. If we or our collaborators have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business, financial condition, results of operations and prospects. If we are unable to successfully identify patients who are likely to benefit from our product candidates or eligible donors, or experience significant delays in doing so, we may not realize the full commercial potential of our product candidates. Trem- cel and any other eHSCs we may develop will require identification of patients that are likely to benefit from administration of our genetically engineered cells in combination with a targeted therapeutic. In addition, ~~VCAR33ALLO~~---**VCAR33** and any other targeted therapeutic we develop will require identification of patients with myeloid malignancies that express specific surface targets and a matched healthy donor. If we, or any third parties that we engage to assist us, are unable to successfully identify such patients or eligible donors, or experience delays in doing so, then:

- our ability to develop any product candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials; and
- we may not realize the full commercial potential of any product candidates we develop that receive marketing approval if, among other reasons, we are unable to appropriately select patients who are likely to benefit from administration of our genetically engineered cells.

Any product candidates we develop may require use of a companion diagnostic to identify patients who are likely to benefit from genetically engineered cell treatment. If safe and effective use of any of our product candidates depends on a companion diagnostic, we may not receive marketing approval, or marketing approval may be delayed, if we are unable to or are delayed in developing, identifying or obtaining regulatory approval or clearance for the companion diagnostic product for use with our product candidate. Identifying a manufacturer of the companion diagnostic and entering into an agreement with the manufacturer could also delay the development of our product candidates. As a result of these factors, we may be unable to successfully develop and realize the commercial potential of our product candidates, and our business, financial condition, results of operations and prospects would be materially adversely affected.

**Risks Related to Our Relationships with Third Parties** We rely on third parties for some aspects of our research and preclinical testing, and we rely on third parties to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research or testing. We rely on third parties to conduct some aspects of our research and preclinical testing, and we rely on third parties, such as CROs, clinical data management organizations, medical institutions such as HCT centers, and clinical investigators, to conduct our clinical trials. Any of these third parties may terminate their engagements with us at any time under certain criteria. If we need to enter into alternative arrangements, it may delay our product development activities. Our reliance on these third parties for research and development and clinical activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA, EMA and other regulatory authorities require us to comply with standards, commonly referred to as Good Clinical Practices, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. In the United States, we also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-

sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. Although we intend to design the future clinical trials for our product candidates, CROs will conduct some or all of the clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct future preclinical studies and clinical trials will also result in less direct control over the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may: • have staffing difficulties; • fail to comply with contractual obligations; • experience regulatory compliance issues; • undergo changes in priorities or become financially distressed; or • form relationships with other entities, some of which may be our competitors. These factors may materially adversely affect the willingness or ability of third parties to conduct our preclinical studies and clinical trials and may subject us to unexpected cost increases that are beyond our control. If the CROs and other third parties do not perform preclinical studies and future clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development, regulatory approval and commercialization of our product candidates may be delayed, we may not be able to obtain regulatory approval and commercialize our product candidates, or our development programs may be materially and irreversibly harmed. If we are unable to rely on preclinical and clinical data collected by our CROs and other third parties, we could be required to repeat, extend the duration of or increase the size of any preclinical studies or clinical trials we conduct and this could significantly delay commercialization and require greater expenditures. Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of our product candidates. We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue. We have initiated manufacturing at our in-house facility, but until and unless we complete the total transfer of our manufacturing capabilities in-house, we will continue to contract with third parties for the manufacture and supply of materials for development of our product candidates and advancement of our current clinical trials, as well as our research programs and preclinical studies, and we expect to continue to do so for future clinical trials and for commercialization of our product candidates. This reliance on third parties increases the risk that we will not have sufficient quantities of such materials, product candidates or any products that we may develop and commercialize, or that such supply will not be available to us at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts. Although we have initiated manufacturing at our in-house manufacturing space in our headquarters, we continue to currently rely on third-party manufacturers, pharmaceutical companies and marrow donor programs, including certain single source suppliers, for the manufacture and supply of materials for development of our product candidates and advancement of our current clinical trial, as well as our research programs and preclinical studies, and expect to continue to do so for future clinical testing and for commercial supply of our product candidates and for which we or our collaborators obtain marketing approval. We do not have a long-term agreement with many of these third-party manufacturers or suppliers, and we frequently purchase our required supply on a purchase order basis. We may be unable to establish any agreements with third-party manufacturers or suppliers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers or suppliers, reliance on third-party manufacturers entails additional risks, including: • the possible breach of the manufacturing or supply agreement by the third party; • the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us; and • reliance on the third party for regulatory compliance, quality assurance, safety and pharmacovigilance and related reporting. Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers or suppliers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocations, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products and harm our business, financial condition, results of operations and prospects. Our product candidates may compete with other product candidates and products for access to manufacturing facilities and other supplies. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Also, prior to the approval of our product candidates, we would need to identify a contract manufacturer that could produce our products at a commercial scale and that could successfully complete FDA pre-approval inspection and inspections by other health authorities. Agreements with such manufacturers or suppliers may not be available to us at the time we would need to have that capability and capacity. Any performance failure on the part of our existing or future manufacturers or suppliers, or any decision by a manufacturer or supplier to remove its products from the market or restrict access to its products, could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant or guaranteed supply for many of the materials we currently use in our preclinical studies and expect to use in our clinical development programs, including for the supply of Mylotarg, donor blood cells, certain apheresis reagents and electroporation machines, and we may have difficulty or be unable to establish alternative sources of these materials. In addition, if any of the manufacturers with whom we have a contractual agreement cannot perform as agreed, we may be

required to replace that manufacturer. Although we believe that there are several potential alternative manufacturers who could replace our contract manufacturers, we may incur added costs and delays in identifying and qualifying any such replacement. Our current and anticipated future dependence upon others for the manufacture of our product candidates and the materials used in our clinical trials may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis. We have and may enter into collaborations with third parties for the research, development and commercialization of our product candidates. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates. We have and may seek third-party collaborators for the research, development and commercialization of certain our product candidates. If we enter into any such arrangements with any third parties in the future, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of collaborations that we have entered into or may enter into in the future. Collaborations involving our current or future product candidates or research programs pose numerous risks to us, including the following:

- Collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations.
- Collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities.
- Collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing.
- Collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours.
- Collaborators with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such products.
- Collaborators may not properly obtain, maintain, enforce or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation.
- Disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources.
- We may lose certain valuable rights under circumstances identified in our collaborations, including if we undergo a change of control.
- Collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.
- Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program under such collaboration could be delayed, diminished or terminated. If our collaborations do not result in the successful development and commercialization of product candidates, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in this Annual Report apply to the activities of our collaborators. These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement 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 several factors. If we license rights to our product candidates, 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. If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans. Our product development and research programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For some of the product candidates, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates. We would face significant competition in seeking appropriate collaborators. Whether we 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 a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA, the EMA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. 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. We may also be restricted under existing collaboration agreements from entering into future agreements on certain terms 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. 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 the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development 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 develop product candidates or bring them to market and generate product revenue.

**Risks Related to Our Intellectual Property**

We are highly dependent on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business. In April 2016, we entered into a license agreement with The Trustees of Columbia University in the City of New York (“Columbia”) pursuant to which we were granted an exclusive, worldwide license to certain intellectual property rights owned or controlled by Columbia, including patents, patent applications, proprietary information, know-how and other intellectual property related to the inhibition of lineage-specific antigens, to develop, commercialize and sell one or more products in any field of use, including related to eHSCs. In addition, in October 2020, we entered into a license agreement with the U. S. Department of Health and Human Services as represented by the National Cancer Institute (“NCI”) of the NIH, pursuant to which we were granted an exclusive, worldwide license to certain intellectual property rights owned or controlled by the NCI, including patents, patent applications, proprietary information, know-how and other intellectual property related to anti-CD33 CAR-T therapies, to develop, commercialize and sell one or more products for the prophylaxis or treatment of CD33-expressing hematological malignancies, including AML and other myeloid malignancies. We are dependent on the patents, know-how and proprietary technology, licensed from Columbia and NCI for the development and, if approved, commercialization of trem-cel and VCAR33, respectively. Any termination of these licenses, or a finding that such intellectual property lacks legal effect, could result in the loss of significant rights and could harm our ability to commercialize our current or future product candidates. Each of the Columbia license agreement and the NCI license agreement imposes certain obligations on us, including obligations to use diligent efforts to meet development thresholds and payment obligations. Non-compliance with such obligations may result in termination of the respective license agreement or in legal and financial consequences. If either Columbia or the NCI terminates its respective license agreement, we may not be able to develop, commercialize or sell our product candidates covered by these agreements. Such an occurrence could materially adversely affect the value of the product candidate being developed under any such agreement or using rights granted under such agreement. Termination of our license agreements or reduction or elimination of our rights under them may result in our having to negotiate a new or reinstated agreement, which may not be available to us on equally favorable terms, or at all, which may mean we are unable to develop, commercialize or sell the affected product candidate or may cause us to lose our rights under the agreement. In August 2023, we entered into a worldwide non-exclusive license from Editas Medicine for ex-vivo Cas9 gene-edited HSC therapies for the treatment and / or prevention of hematological malignancies. The license provides access to key intellectual property for the continued development and commercialization of edited HSCs including trem-cel, with the option to elect additional product candidate targets within the next five years. Failure to maintain this license, or obtain a replacement license for our field of use on commercially reasonable terms or at all, could harm our ability to commercialize our current or future product candidates. In addition, our licensors may make decisions in prosecuting, maintaining, enforcing and defending any licensed intellectual property rights, for example, any licensed patents or patent applications, that may not be in our best interest. Moreover, if our licensors take any action with respect to any licensed intellectual property rights, for example, any licensed patents or patent applications, that results in a successful challenge to the licensed intellectual property by a third party, such patents may be invalidated or held to be unenforceable, and we may lose our rights under such patents, which could materially harm our business. Further, the agreements under which we currently license intellectual property from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. Accordingly, disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including those relating to:

- the scope of rights, if any, granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the license agreement;
- whether our licensor or its licensor had the right to grant the license agreement;
- whether third parties are entitled to compensation or equitable relief, such as an injunction, for our use of the intellectual property without their authorization;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- whether we are complying with our obligations with respect to the use of the licensed technology in relation to our development and commercialization of product candidates;
- our involvement in the prosecution and enforcement of the licensed patents and our licensors’ overall patent prosecution and enforcement strategy;
- the allocation of ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and by us and any future partners or collaborators; and
- the amounts of royalties, milestones or other payments due under the license agreement.

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. If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, or are insufficient to provide us the necessary rights to use the intellectual property, we may be unable to successfully develop and commercialize the affected product candidates. If we or any of our licensors fail to adequately protect this intellectual property, our ability to commercialize our products could suffer. Any disputes with our licensors or any termination of the licenses on which we depend could have a material adverse effect on our business, financial condition, results of operations and prospects. Our commercial success depends on our ability to obtain, maintain and protect our

intellectual property and proprietary technology. Our commercial success depends in large part on our ability to obtain, maintain and protect intellectual property rights through patents, trademarks and trade secrets in the United States and other countries with respect to our proprietary product candidates. If we do not adequately protect our intellectual property rights, competitors may be able to erode, negate or preempt any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we own and have in- licensed certain intellectual property rights, including certain issued patents and patent applications, and have filed and may file provisional and non- provisional patent applications in the United States or abroad related to our product candidates that are important to our business. Provisional patent applications are not eligible to become issued patents until, among other things, we file a non- provisional patent application within 12 months of the filing of one or more of our related provisional patent applications. If we do not timely file non- provisional patent applications, we may lose our priority date with respect to our provisional patent applications and any patent protection on the inventions disclosed in our provisional patent applications. 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. Moreover, the patent application and approval process is expensive and time- consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. The patent application, prosecution and enforcement processes are subject to numerous risks and uncertainties, and there can be no assurance that we, our licensors, or any of our current or future collaborators will be successful in protecting our product candidates by obtaining, defending and / or asserting patent rights. These risks and uncertainties include the following: • the U. S. Patent and Trademark Office (the “ USPTO ”) and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case; • patent applications may not result in any patents being issued; • patents that may be issued or in- licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage; • our competitors, many of whom have substantially greater resources and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use, and sell our potential product candidates; • there may be significant pressure on the U. S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and • countries other than the United States may have patent laws less favorable to patentees than those upheld by U. S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates. In some instances, agreements through which we license intellectual property rights may not give us control over patent prosecution or maintenance, so that we may not be able to control which claims or arguments are presented, how claims are amended, and may not be able to secure, maintain, or successfully enforce necessary or desirable patent protection from those patent rights. We have not had and do not have primary control over patent prosecution and maintenance for certain of the patents and patent applications we license, including under our license agreements with Columbia and NCI, and therefore cannot guarantee that these patents and applications will be prosecuted or maintained in a manner consistent with the best interests of our business. We cannot be certain that patent prosecution and maintenance activities by our licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents. Moreover, some of our in- licensed patents and patent applications may be, and some of our future owned and licensed patents 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. The patent protection we obtain for our product candidates may not be sufficient to provide us with any competitive advantage or our patents may be challenged. Our owned and licensed patents and pending patent applications, if issued, may not provide us with any meaningful protection or may not prevent competitors from designing around our patent claims to circumvent our patents by developing similar or alternative technologies or therapeutics in a non- infringing manner. For example, a third party may develop a competitive product that provides benefits similar to one or more of our product candidates but falls outside the scope of our patent protection or license rights. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidates could be negatively affected, which would harm our business. Currently, a significant portion of our patents and patent applications are in- licensed, though similar risks would apply to any patents or patent applications that we now own or may own or in- license in the future. It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If we or our partners, collaborators, licensees, or licensors, whether current or future, fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our partners, collaborators, licensees, or licensors, are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and / or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business. In addition, the determination of patent rights with respect to clinical compositions of matter and treatment methods commonly involves complex legal and factual

questions, which are dependent upon the current legal and intellectual property context, extant legal precedent and interpretations of the law by individuals. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are characterized by uncertainty. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, patent laws in various jurisdictions, including significant commercial markets such as Europe, restrict the patentability of methods of treatment of the human body more than U. S. law does. If these changes were to occur, they could have a material adverse effect on our ability to generate revenue. 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. Assuming the other requirements for patentability are met, currently, the first party to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States the first party to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that 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. Similarly, we cannot be certain that parties from whom we do or may license or purchase patent rights were the first to make relevant claimed inventions, or were the first to file for patent protection for them. If third parties have filed prior patent applications on inventions claimed in our patents or applications that were filed on or before March 15, 2013, an interference proceeding in the United States can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such prior applications after March 15, 2013, a derivation proceeding in the United States can be initiated by such third parties to determine whether our invention was derived from theirs. Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our owned and licensed patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found. If such prior art exists, it may be used to invalidate a patent, or may prevent a patent from issuing from a pending patent application. For example, such patent filings may be subject to a third-party submission of prior art to the USPTO, or to other patent offices around the world. Alternately or additionally, we may become involved in post- grant review procedures, oppositions, derivation proceedings, ex parte reexaminations, inter partes review, supplemental examinations, or interference proceedings or challenges in district court, in the United States or in various foreign patent offices, including both national and regional, challenging patents or patent applications in which we have rights, including patents on which we rely to protect our business. An adverse determination in any such challenges may result in loss of the patent or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, or in denial of the patent application or loss or reduction in the scope of one or more claims of the patent application, any of 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. Issued patents that we have or may obtain or license may not provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products, for example, by submitting a Section 351 (k) Biologics License Application ("BLA") to the FDA, or pursue similar strategies in the United States or other jurisdictions, in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors are competing in a non- infringing manner. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects. Other parties have developed or may develop technologies that may be related to or competitive with our approach, and may have filed or may file patent applications and may have been issued or may be issued patents with claims that overlap or conflict with our patent applications, either by claiming the same materials, formulations or methods, or by claiming subject matter that could dominate our patent position. In addition, certain parts or all of the patent portfolios licensed to us are, or may be, licensed to third parties and such third parties may have or may obtain certain enforcement rights. If the scope of the patent protection we or our licensors obtain is not sufficiently broad, we may not be able to prevent others from developing and commercializing technology and products similar or identical to ours. The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our licensed patents have, or that any of our pending owned or licensed patent applications that mature into issued patents will include, claims with a scope sufficient to protect our product candidates or otherwise provide any competitive advantage, nor can we provide any assurance that our licenses will remain in force. If we are unable to protect the confidentiality of our trade secrets, our business and competitive position may be harmed. In addition to the protection afforded by patents, we rely upon trade secret protection, know- how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our contractors, collaborators, scientific advisors, employees and consultants and invention assignment agreements with our

consultants and employees. However, we may not obtain these agreements in all circumstances, and individuals with whom we have these agreements may not comply with their terms. The assignment of intellectual property rights under these agreements 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. In addition, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements despite the existence of confidentiality agreements and other contractual restrictions. Monitoring unauthorized uses and disclosures is difficult and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the contractors, collaborators, scientific advisors, employees and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets. Enforcing a claim against a third party that illegally obtained and is using our trade secrets, like patent litigation, is expensive and time-consuming and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing or unwilling to protect trade secrets. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, our trade secrets could otherwise become known or be independently discovered by our competitors or other third parties. Competitors and other third parties could attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. 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, or those to whom they communicate it, from using that technology or information to compete with us. If our trade secrets are not adequately protected or sufficient to provide an advantage over our competitors, our competitive position could be adversely affected, as could our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets. We may not be successful in acquiring or in-licensing necessary rights to key technologies underlying our product candidates. We currently have rights to intellectual property, through licenses from third parties, to develop our product candidates, and we expect to seek to expand our intellectual property footprint related to our product candidate pipeline in part by in-licensing the rights to key technologies. The future growth of our business will depend in part on our ability to in-license or otherwise acquire the rights to develop additional product candidates and technologies. Although we have succeeded in licensing technologies from third party licensors, including Columbia and NCI, in the past, we can give no assurance that we will be able to in-license or acquire the rights to other technologies relevant to our product candidates from third parties on acceptable terms or at all. In order to market our product candidates, we may find it necessary or prudent to obtain licenses from such third party intellectual property holders. However, it may be unclear who owns the rights to intellectual property we wish to obtain, or we may be unable to secure such licenses or otherwise acquire or in-license intellectual property rights from third parties that we identify as necessary for our product candidates and technology we employ. For example, we employ a range of genome engineering technologies that are owned by third parties in our preclinical studies, as well as to manufacture the supply of eHSCs or other cell therapies used for clinical trials and, if approved, for commercialization of our product candidates. In particular, we rely on, and will continue to rely on, CRISPR-Cas9 genome engineering technology to create trem-cel. We currently conduct our preclinical research and clinical trials under 35 U. S. C. § 271 (e) (1), which provides a safe harbor from patent infringement for uses of patented technology reasonably related to the development and submission of information under a federal law which regulates the manufacture, use, or sale of drugs. While we have secured a worldwide non-exclusive license from Editas Medicine for ex vivo Cas9 gene-edited HSC therapies for the continued development and potential commercialization of edited HSCs including trem-cel, with the option to elect additional product candidate targets within the next five years, failure to maintain this license, or obtain a replacement license for our field of use on commercially reasonable terms or at all, could harm our ability to commercialize our current or future product candidates. Numerous patents and patent applications directed to genome engineering technology have been filed by third parties. For example, we are aware of a number of patents and patent applications by the University of California, the University of Vienna, and Emmanuelle Charpentier; the Broad Institute, Inc.; the Massachusetts Institute of Technology; the Presidents and Fellows of Harvard College; Sigma-Aldrich Co.; Novartis AG; Vilnius University; Agilent Technologies, Inc.; Collectis; Sangamo Therapeutics, Inc; The Trustees of Princeton University; Miltenyi Biotec GmbH (“Miltenyi”); Amgen Research (Munich) GmbH; and the University of Pennsylvania, among others. The intellectual property space related to genome engineering, particularly with respect to CRISPR-Cas9, is highly complex and still unsettled. For example, certain CRISPR-Cas9 patents of various parties previously mentioned above are currently subject to interference proceedings before the USPTO and opposition proceedings before the European Patent Office. It is uncertain when and how the USPTO as well as the European Patent Office will decide in the various proceedings, and the decisions of the respective patent offices may significantly affect the scope or may deny the validity of the respective patents involved in these proceedings. Although we recently obtained a license to genome engineering technology, including to CRISPR-Cas9 technology from Editas Medicine, we may be required to obtain licenses from more than one party, or from different parties in different parts of the world. In certain scenarios, it may also be difficult or impossible, at least for a certain time, to identify whether a license, if available at all, would convey sufficient intellectual property rights to us that would allow us to avoid third-party claims of intellectual property infringement, misappropriation or other violations. The licensing or acquisition of third party intellectual property rights is a highly competitive area, and other companies may pursue strategies to license or acquire third party intellectual property rights that we may consider attractive or necessary. Such companies may have a competitive advantage over us, e. g., 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. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on

our investment or at all. 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 program or product candidate, which could have a material adverse effect on our business, financial condition, results of operations and prospects. Even if we were able to obtain such a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, we may be unable to commercialize our product candidates or such commercialization efforts may be significantly delayed, which could in turn significantly harm our business. Third-party claims of intellectual property infringement, misappropriation or other violations may prevent or delay our product discovery and development efforts and have a material adverse effect on our business. Our commercial success depends in part on our avoiding infringement, misappropriation and other violations of the patents and proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. Recently, under U. S. patent reform, new procedures including inter partes review and post grant review have been implemented. This reform will bring uncertainty to the possibility of challenge to our patents in the future. Numerous U. S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates, and third parties may allege they have patent rights encompassing our product candidates, technologies or methods. Third parties may assert that we are employing their proprietary technology without authorization and may file patent infringement claims or lawsuits against us, and if we are found to infringe such third-party patents, we may be required to pay damages, cease commercialization of the infringing technology or obtain a license from such third parties, which may not be available on commercially reasonable terms or at all. We are aware of several third-party patents and patent applications that if issued, may be construed to cover eHSC technology. For example, Miltenyi's European patent EP3025719 covers technology related to eHSC products. This patent was subject to opposition proceedings before the European Patent Office Opposition Division (the "Opposition Division") and in March 2021, the Opposition Division revoked the patent. This decision was appealed and reviewed before the Board of Appeal of the European Patent Office. The appeal was subsequently withdrawn, and the patent revoked. Miltenyi also has several issued U. S. patents related to eHSC technology. In addition, the University of Pennsylvania has filed patent applications and has been granted several U. S. and foreign patents covering eHSC technology. These or other third parties owning or controlling patent rights may seek to allege that our development and commercialization of our eHSC products, including trem-cel, infringes such patent rights and file a patent infringement lawsuit against us in the future. While we believe that we have valid defenses against any such allegation or lawsuit, such defenses may ultimately be unsuccessful. There may also be third-party patents of which we are currently unaware with patent rights to materials, formulations, methods of manufacture or methods of treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Further, we or our licensors may fail to identify even those relevant third-party patents that have issued or may incorrectly interpret the relevance, scope or expiration of such patents. 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 scope of a patent or a pending application may be incorrect. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our product candidates, materials used in or formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize the product candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our materials, formulations or methods, including without limitation, combination therapy or patient selection methods, the holders of any such patent may be able to block our ability to develop and commercialize the product candidate unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable. Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would involve a substantial diversion of employee resources from our business. We may not have sufficient resources to bring these actions to a successful conclusion, which may result in significant cost and may impede our inability to pursue any affected products or product candidates. 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. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, 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 on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse 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. Noncompliance 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. In such an event, our competitors might be able to enter the market earlier than would otherwise have been the case, which would have a material adverse effect on our business. Some intellectual property that we have licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as “march-in” rights, certain reporting requirements and a preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights and limit our ability to contract with non-U.S. manufacturers. Any of the intellectual property rights that we have licensed or we may license in the future and that have been generated through the use of U.S. government funding are subject to certain federal regulations. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980 (the “Bayh-Dole Act”). These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any such intellectual property rights to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as “march-in rights”). The U.S. government also has the right to take title to such intellectual property rights if we, or the applicable licensor, fail to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. We cannot be certain that our current or future licensors will comply with the disclosure or reporting requirements of the Bayh-Dole Act at all times, or be able to rectify any lapse in compliance with these requirements. In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply. 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 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. 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 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. 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, there can be no assurance 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. Any of the foregoing may have a material adverse effect on our business, financial condition, results of operations and prospects. Changes to 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. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the Congress, the

federal courts, and the USPTO, 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 case, *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.*, the Supreme Court held that certain claims to DNA molecules are not patentable. In addition, the case *Amgen Inc. v. Sanofi* affects the way antibody claims are examined and litigated. While we do not believe that any of the patents owned or licensed by us will be found invalid based on these decisions, we cannot predict how future decisions by the courts, the Congress or the USPTO may impact the value of our patents. We may not be able to protect our intellectual property rights throughout the world. Filing, prosecuting, maintaining, defending and enforcing patents on our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, 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 drugs and may export otherwise infringing drugs to territories where we have patent protection, but enforcement rights are not as strong as those in the United States. These drugs may compete with our product candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of some countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents 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 lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Many countries have compulsory licensing laws under which a patent owner may be compelled under specified circumstances to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. 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, which could adversely affect 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. Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest filing date of a non-provisional application to which the patent claims priority. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product candidate, we may be open to competition from competitive medications, including generic medications. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing product candidates similar or identical to ours. If we do not obtain patent term extension and data exclusivity for trem-cel or any other product candidates, our business may be materially harmed. Depending upon the timing, duration and conditions of any FDA marketing approval of our product candidates, one or more of our U. S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch- Waxman Amendments, and similar legislation in the European Union. The Hatch- Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. However, we may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. Only one patent per approved product can be extended, the extension cannot extend the total patent term beyond 14 years from approval and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for the applicable product candidate will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced. Further, if this occurs, our competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case, and our competitive position, business, financial condition, results of operations, and prospects could be materially harmed. Third parties may assert that our employees or consultants have wrongfully used or disclosed confidential information or misappropriated trade secrets. We employ individuals who were previously employed at universities or other biopharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of a former employer or other third parties. We may also be subject to claims that patents and applications that we may file to protect inventions of our employees or consultants are rightfully owned by their former employers or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages,

we may lose valuable intellectual property rights or personnel. 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. Any of the foregoing would harm our business, financial condition, results of operations, and prospects. 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 registered or unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. 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. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our business, financial condition, results of operations and growth prospects. Intellectual property rights do not necessarily address all potential threats. 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:

- Our product candidates may eventually become available in generic or biosimilar product forms;
- others may be able to make products that are similar to our product candidates or utilize similar technology but that are not covered by the claims of the patents that we license or own;
- we, or our current or future licensors might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or own;
- we, or our current or future licensors might not have been the first to file patent applications covering certain of our or their inventions;
- we, or our license partners or current or future collaborators, may fail to meet our obligations to the U. S. government regarding any in-licensed patents and patent applications funded by U. S. government grants, leading to the loss or unenforceability of patent rights;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending, owned or licensed patent applications or those that we may own in the future will not lead to issued patents;
- it is possible that there are prior public disclosures that could invalidate our owned or in- licensed patents, or parts of our owned or in- licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our product candidates or technology similar to ours;
- it is possible that our owned or in- licensed patents or patent applications omit individual (s) that should be listed as inventor (s) or include individual (s) that should not be listed as inventor (s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable;
- it is possible that our pending owned or licensed patent applications or those that we may own or license in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- the claims of our owned or in- licensed issued patents or patent applications, if and when issued, may not cover our product candidates;
- the laws of foreign countries may not protect our proprietary rights or the proprietary rights of license partners or current or future collaborators to the same extent as the laws of the United States;
- the inventors of our owned or in- licensed patents or patent applications may become involved with competitors, develop products or processes that design around our patents or become hostile to us or the patents or patent applications on which they are named as inventors;
- 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 have engaged in scientific collaborations in the past and will continue to do so in the future and our collaborators may develop adjacent or competing products that are outside the scope of our patents;
- we may not develop additional proprietary technologies that are patentable;
- any product candidates we develop may be covered by third parties' patents or other exclusive rights;
- the patents of others may harm our business; and
- 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.

Should any of these events occur, they could harm our business, financial condition, results of operations, and prospects. Risks Related to Regulatory and Other Legal Compliance Matters Failure to obtain marketing approval in foreign jurisdictions would prevent product candidates from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue. In order to market and sell product candidates in the European Union and other foreign jurisdictions, we or our third- party collaborators must obtain separate marketing approvals (a single one for the European Union) and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product candidate be approved for reimbursement before the product candidate can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any jurisdiction, which would materially impair our ability to generate revenue. Even if we, or any collaborators we may have, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our product candidates could require the substantial expenditure of resources and may limit how we, or they, manufacture and market our product candidates, which could materially impair our

ability to generate revenue. Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post- approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA, EMA, the Competent Authorities of the Member States of the European Union and other regulatory authorities. These requirements include submissions of safety and other post- marketing information and reports, facility registration and drug listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for costly post- marketing testing and surveillance to monitor the safety or efficacy of the product. The FDA and other regulatory authorities may restrict the use of our products to certain specialists and / or institutions and require formal reporting and approval of a REMS program. Such restrictions or requirements could deter use of our products by certain individuals or institutions. Accordingly, assuming we, or any of our collaborators, receive marketing approval for one or more product candidates, we, such collaborators and our and their contract manufacturers will continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control. If we and such collaborators are not able to comply with post- approval regulatory requirements, we and such collaborators could have the marketing approvals for our products withdrawn by regulatory authorities and our, or such collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post- approval regulations may have a negative effect on our business, operating results, financial condition and prospects. **Any Even if we receive regulatory approval for any product candidate, we will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product candidates** for which we obtain marketing approval could be subject to **labeling and other** restrictions or withdrawal from the market, and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved. **If the FDA or a comparable foreign regulatory authority approves any of our product candidates, the manufacturing processes, testing, safety, efficacy, labeling, packaging, distribution, import, export, 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, as well as continued compliance with cGMPs and GCPs for any clinical trials that we conduct post- approval, all of which may result in significant expense and limit our ability to commercialize such products. In addition, any regulatory approvals that we receive for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post- marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate.** The FDA, the EMA, the Competent Authorities of the Member States of the European Union and other regulatory **agencies authorities** closely regulate the post- approval marketing and promotion of products to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA, the Competent Authorities of the Member States of the European Union and other regulatory **agencies authorities** impose stringent restrictions on manufacturers' communications regarding off- label use, and if we market our products for off- label use, we may be subject to enforcement action for off- label marketing by the FDA and other federal and state enforcement agencies, including the Department of Justice. While physicians may prescribe products for off- label uses as the FDA and other U. S. regulatory agencies do not regulate a physician' s choice of drug treatment made in the physician' s independent medical judgment, they do restrict promotional communications from companies or their sales force with respect to off- label uses of products for which marketing clearance has not been issued. Companies may only share truthful and not misleading information that is otherwise consistent with a product' s FDA approved labeling. Violation of the Federal Food, Product, and Cosmetic Act and other statutes, including the False Claims Act and equivalent legislation in other countries relating to the promotion and advertising of prescription products may also lead to investigations or allegations of violations of federal and state and other countries' health care fraud and abuse laws and state consumer protection laws. Even if it is later determined we were not in violation of these laws, we may be faced with negative publicity, incur significant expenses defending our actions and have to divert significant management resources from other matters. In addition, later discovery of previously unknown problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various negative consequences, including: • restrictions on such products, manufacturers or manufacturing processes; • restrictions on the labeling or marketing of a product; • restrictions on the distribution or use of a product; • requirements to conduct post- marketing clinical trials; • receipt of warning or untitled letters; • withdrawal of the products from the market; • refusal to approve pending applications or supplements to approved applications that we submit; • recall of products; • fines, restitution or disgorgement of profits or revenue; • restrictions on future procurements with governmental authorities; • suspension or withdrawal of marketing approvals; • suspension of any ongoing clinical trials; • refusal to permit the import or export of our products; • product seizure; and • injunctions or the imposition of civil or criminal penalties. 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 ability to commercialize any of our product candidates, if approved, and adversely affect our business, financial condition, results of operations and prospects. **Moreover, the policies of the FDA and of comparable foreign regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. For example, the U. S. Supreme Court' s June 2024 decision in Loper Bright Enterprises v. Raimondo overturned the longstanding Chevron doctrine, under which courts were required to give deference to regulatory agencies' reasonable**

**interpretations of ambiguous federal statutes. The Loper decision could result in additional legal challenges to regulations and decisions issued by federal agencies, including the FDA, on which we rely. Any such legal challenges, if successful, could have a material impact on our business. Additionally, the Loper decision may result in increased regulatory uncertainty, inconsistent judicial interpretations, and other impacts to the agency rulemaking process, any of which could adversely impact our business and operations. We 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. 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 be subject to enforcement action and we may not achieve or sustain profitability.**

Disruptions at the FDA and other government agencies caused by funding shortages, layoffs, shifting priorities under the new administration 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, 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, layoffs, 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 or modifications to cleared or approved biologics to be reviewed and / or approved by necessary government agencies, which would adversely affect our business. ~~For example~~ **In addition, the Trump administration has discussed several changes to the reach and oversight of the FDA, which could affect its relationship with the pharmaceutical industry, transparency in decision making and ultimately the cost and availability of prescription drugs. Additionally,** over the last several years, ~~including for 35 days beginning on December 22, 2018,~~ the U. S. government has shut down several ~~multiple~~ times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA **and other government** employees and stop critical activities. **If funding for the FDA is reduced, FDA priorities change, or a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.** Our relationships with healthcare providers, including physicians, and third-party payors will be subject to applicable anti-kickback, fraud and abuse, health data privacy, transparency, anti-bribery and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings. Healthcare providers and third-party payors play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our current and future arrangements with healthcare providers, third-party payors and customers may expose us to broadly applicable federal and state fraud and abuse, transparency, health data privacy, and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we research as well as market, sell and distribute our products for which we obtain marketing approval. ~~If we are found to be in violation of any of any healthcare laws or any other federal or state regulations, we may be subject to significant administrative, civil and / or criminal penalties, damages, fines, disgorgement, imprisonment, exclusion from federal health care programs, additional reporting requirements and / or oversight, and the curtailment or restructuring of our operations.~~ Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Given the breadth of the laws and regulations and evolving government interpretations of the laws and regulations, governmental authorities may possibly conclude that our business practices, including certain of our advisory board arrangements with physicians, some of whom are compensated in the form of stock or stock options, may not comply with healthcare laws and regulations. If our operations are found to be in violation of any **healthcare** of the laws described above or any other **federal or state** government regulations that apply to us, we may be subject to significant penalties, including administrative, civil and criminal penalties, damages, fines, disgorgement, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment, integrity oversight and reporting obligations, contractual damages, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our business, financial condition, results of operations, and prospects. For a more detailed discussion of U. S. healthcare laws that may affect our business, see "**Business — Other U. S. Healthcare Laws and Regulations Compliance Requirements**" in Part I, Item 1 of this Annual Report.

Healthcare and other reform legislation, may increase the difficulty and cost for us and any collaborators we may have to obtain marketing approval of and commercialize our product candidates, if approved, and affect the prices we, or they, may obtain. In the United States and some foreign jurisdictions, there have been and continue to be ongoing efforts to implement legislative and regulatory changes regarding the healthcare system. Such changes could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidates for which we obtain marketing approval. Although we cannot predict what healthcare or other reform efforts will be successful, such efforts may result in more rigorous coverage criteria, in additional downward pressure on the price that we, or our future collaborators, may receive for any approved products or in other consequences that may adversely affect our ability to achieve or maintain profitability. Within the United States, the federal government and individual states have aggressively pursued healthcare reform, as evidenced by the passing of the ACA. The ACA substantially changed the way healthcare is financed by both governmental and private insurers and contains a number of provisions that affect coverage and reimbursement of drug products and / or that could potentially reduce the demand for pharmaceutical products. There have been executive, judicial and Congressional challenges **and amendments**, to certain aspects of the ACA. For example, ~~on June 17, 2021, the United States Supreme Court dismissed a challenge on procedural grounds that argued the ACA is unconstitutional in its entirety because the~~

“individual mandate” was repealed by Congress. In addition, on August 16, 2022, President Biden signed the Inflation Reduction Act of 2022, or IRA, **was signed** into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA also eliminates the “donut hole” under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and creating a new manufacturer discount program. It is possible that the ACA will be subject to judicial or Congressional challenges in the future. We cannot predict the ultimate content, timing or effect of any such challenges or changes to the ACA or other federal and state reform efforts. There is no assurance that federal or state health care reform will not adversely affect our future business and financial results, and we cannot predict how future federal or state legislative, judicial or administrative changes relating to healthcare reform will affect our business. Federal and state governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs. **At For example, the IRA also, among the other things** federal level, **(1) directs** in July 2021, the Biden administration released an executive order that included multiple provisions aimed at prescription drugs. In response to Biden’s executive order, on September 9, 2021, the U. S. department **Department** of Health and Human Services (“HHS”) released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform. The plan sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. In addition, the IRA also, among other things, **(1) directs** HHS to negotiate the price of certain **high expenditure** single-source drugs and biologics **that have been on the market for at least 11 years** covered under Medicare **(the “Medicare Drug Price Negotiation Program”)**, and **(2) imposes** rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions **began to** take effect progressively **starting** in fiscal year 2023. On August **29-15, 2023-2024**, HHS announced the **list agreed-upon price** of the first ten **drugs products** that **were will be** subject to price negotiations, although the Medicare **Drug Price Negotiation Program** is currently subject to legal challenges. **It is unclear how the IRA** **On January 17, 2025, HHS selected fifteen additional products covered under Part D for price negotiation in 2025. Each year thereafter more Part B and Part D products will become subject** be implemented but is likely to have a significant impact on the pharmaceutical industry. In response to the Biden administration’s October 2022 executive order, on February 14, 2023, HHS released a report outlining three **the Medicare Drug Price Negotiation Program** new models for testing by the CMS Innovation Center which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. **It is unclear whether the models will be utilized in any health reform measures in the future.** Further, on December 7, 2023, the Biden administration **announced** an initiative to control the price of prescription drugs through the use of march-in rights under the Bayh-Dole Act **was announced**. On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework. We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third party coverage and reimbursement may be enacted in the future, **particularly in light of the recent U. S. Presidential and Congressional elections**, or what effect such legislation, regulations or policies would have on our business. Any cost containment measures could significantly decrease the available coverage and the price we might establish for our potential products, which would have an adverse effect on our net revenues and operating results. For a more detailed discussion of U. S. healthcare reforms that may affect our business, see “Business — Healthcare Reform” in Part I, Item 1 of this Annual Report. 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, consultants and commercial partners, and, if we commence clinical trials, our principal investigators. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the EMA and other regulatory authorities, 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 restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA, the EMA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but 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 government 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, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations, and prospects, including the imposition of significant fines or other sanctions. Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and require us to develop and implement costly compliance programs. We may be subject to numerous laws and regulations in each jurisdiction outside the United States in which we may operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required. The Foreign Corrupt Practices Act (the “FCPA”) prohibits any U. S. individual or business from paying, offering, 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 certain accounting provisions requiring the company 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. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The SEC is involved with enforcement of the books and records provisions of the FCPA. Similarly, the U. K. Bribery Act 2010 has extra-territorial effect for companies and individuals having a connection with the United Kingdom. The U. K. Bribery Act prohibits inducements both to public officials and private individuals and organizations. Compliance with the FCPA and the U. K. Bribery Act is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions. Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U. S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. Our expansion outside of the United States has required, and will continue to require, us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing or selling certain product candidates outside of the United States, which could limit our growth potential and increase our development costs. The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U. S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U. S. exchanges for violations of the FCPA's accounting provisions. We **and the third parties with whom we work** are subject to stringent and evolving privacy and information security laws, regulations, industry standards, policies ~~and~~, contractual obligations and **other obligations related to privacy and information security. Our (our - or the third parties with whom we work)** actual or perceived failure to comply with such obligations could adversely affect our business. In the ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, "process") personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, sensitive third-party data, business plans, transactions, information about patients and clinical trial data (collectively, sensitive data). Our data processing activities subject us to numerous data privacy and security laws and regulations, which among other things, impose certain requirements relating to the privacy, security and transmission of personal ~~information~~ **data**. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. ~~Actual or perceived Failure-failure~~ to comply with any of these laws and regulations could result in enforcement action against us, including fines, imprisonment of company officials and public censure, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects. In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e. g., Section 5 of the Federal Trade Commission Act), and other similar laws (e. g., wiretapping laws). For example, the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), imposes specific requirements relating to the privacy, security, and transmission of individually identifiable protected health information. In addition, ~~in the past few years,~~ numerous U. S. states —including California, Virginia, Colorado, Connecticut, and Utah—have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020 (collectively, "CCPA"), applies to personal data of consumers, business representatives, and employees who are California residents, and requires businesses to provide specific disclosures in privacy notices and honor requests of such individuals to exercise certain privacy rights. Although there are minimum revenue thresholds for companies to be subject to these laws and there are limited exemptions for clinical trial data under the CCPA and similar state comprehensive privacy laws, such laws may impact (possibly significantly) our business activities depending on how they are interpreted, should we become subject to the CCPA or such state comprehensive privacy laws in the future. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future. These developments may further complicate compliance efforts and increase legal risk and compliance costs for us and the third parties ~~upon~~ **with whom we rely work. Our employees and personnel use generative artificial intelligence ("AI") technologies to perform their work, and the disclosure and use of personal data in generative AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating generative AI. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and lawsuits. If we are unable to use generative AI, it could make our business less efficient and result in competitive disadvantages.** In addition to data privacy and security laws, we are bound by other contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful.

**Regulators are increasingly scrutinizing these** ~~We also publish privacy policies, marketing materials, and other statements, regarding data privacy and security~~ and if these policies, materials, or statements are found to be deficient, lacking in transparency, deceptive, unfair, **misleading**, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, or other adverse consequences. Obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing, becoming increasingly stringent, and creating uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties **on with** whom we **rely work** may fail to comply with such obligations, which could negatively impact our business operations. If we are unable to properly protect the privacy and security of sensitive data in our possession, we could be found to have breached our contracts. Further, if we fail to comply with applicable privacy and security laws, including applicable HIPAA privacy and security standards, we could face significant consequences, including but not limited to: government enforcement actions (e. g., administrative, civil and criminal penalties, investigations, audits, inspections, and similar); litigation (including class- action claims); additional reporting requirements and / or oversight; bans on processing personal data (including clinical trial data); and orders to destroy or not use personal data. In addition, our ongoing efforts to comply with evolving **privacy and data security** laws and regulations **have been at the federal and state level** may **in the future** be costly and require ongoing modifications to our policies, procedures and systems.

**Risks Related to Employee Matters, Managing Growth and Information Technology** Our future success depends on our ability to retain our Chief Executive Officer and other key executives and to attract, retain and motivate qualified personnel. We are highly dependent on Robert Ang, M. B. B. S., M. B. A., our Chief Executive Officer, as well as the other principal members of our management and scientific teams. Dr. Ang and such other principal members are employed "at will," meaning we or they may terminate the employment at any time. We do not maintain "key person" insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives. Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our product candidates toward scaling up for commercialization, manufacturing and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors, including our scientific founder, may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. The inability to recruit, or loss of services of certain executives, key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives and have a material adverse effect on our business, financial condition, results of operations and prospects. We expect to expand our development, regulatory and future sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations. In connection with the growth and advancement of our pipeline, we expect to increase the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs, manufacturing and, as our product candidates advance through later stages of clinical development, sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expected expansion of our operations or recruit and train additional qualified personnel. Moreover, the expected physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations. As a growing biotechnology company, we are actively pursuing new platforms and product candidates in many therapeutic areas and across a wide range of diseases. Successfully developing product candidates for and fully understanding the regulatory and manufacturing pathways to all of these therapeutic areas and disease states requires a significant depth of talent, resources and corporate processes in order to allow simultaneous execution across multiple areas. Due to our limited resources, we may not be able to effectively manage this simultaneous execution and the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure, give rise to operational mistakes, legal or regulatory compliance failures, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of our product candidates. If our management is unable to effectively manage our expected development and expansion, our expenses may increase more than expected, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to compete effectively and commercialize our product candidates, if approved, will depend in part on our ability to effectively manage the future development and expansion of our company. Our insurance policies may be inadequate and potentially expose us to unrecoverable risks. We have limited director and officer insurance and commercial insurance policies. Any significant insurance claims would have a material adverse effect on our business, financial condition and results of operations. Insurance availability, coverage terms and pricing continue to vary with market conditions. We endeavor to obtain appropriate insurance coverage for insurable risks that we identify; however, we may fail to correctly anticipate or quantify insurable risks, we may not be able to obtain appropriate insurance coverage, and insurers may not respond as we intend to cover insurable events that may occur. We have observed rapidly changing conditions in the insurance markets relating to nearly all areas of traditional corporate insurance. Such conditions have resulted in higher premium costs, higher policy deductibles and lower

coverage limits. For some risks, we may not have or maintain insurance coverage because of cost or availability. If our information technology systems, or those of our third- party vendors, collaborators, or other contractors or consultants **or other third parties with whom we work**, or our data are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to a material disruption of our product development programs, regulatory investigations or actions, litigation, fines and penalties, reputational harm and other adverse consequences. In the ordinary course of our business, we and the third parties ~~upon which~~ **with whom** we ~~rely~~ **work** process sensitive data, and, as a result, we ~~such and the third parties upon which we rely~~ face a variety of evolving threats that could cause security incidents. Our information technology systems and those of our current and any future third- party vendors, collaborators ~~and~~, **consultants or other contractors or consultants** ~~third parties with whom we work~~ are subject to damage or interruption from a variety of evolving threats, including but not limited to social- engineering attacks (including through deep fakes, which ~~are~~ **may be** increasingly more difficult to identify as fake, and phishing attacks), computer viruses, computer hackers, malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), employee theft or misuse, denial- of- service attacks, credential stuffing, credential harvesting, ransomware attacks, adware, attacks enhanced or facilitated by AI, sophisticated nation- state and nation- state- supported actors, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments. Remote work ~~has become more common and~~ has increased risks to our information technology systems and data, as more of our employees utilize network connections, computers, and devices outside our premises or network, including working at home, while in transit and in public locations. Additionally, future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program. While we seek to protect our information technology systems from system failure, accident and security breach, if such an event were to occur and cause interruptions in our operations, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other sensitive data or other disruptions. For example, the loss of clinical trial data from ~~future~~ **completed, ongoing or planned** clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If we were to experience a significant cybersecurity breach of our information systems or sensitive data, the costs associated with the investigation, remediation and potential notification of the breach to counterparties and data subjects could be material. In addition, our remediation efforts may not be successful. If we are unable to build and sustain the proper technology and cybersecurity infrastructure, we could suffer significant business disruption, including transaction errors, supply chain or manufacturing interruptions, processing inefficiencies, or the loss of or damage to sensitive data. Although we have implemented security measures designed to help protect sensitive data from unauthorized access or disclosure, our information technology and infrastructure may be vulnerable in the future to attacks by hackers or viruses, failures, or breaches due to third- party action, employee negligence or error, malfeasance, or other incidents or disruptions. For example, we ~~could be~~ **have been** the target of phishing attacks ~~seeking confidential information regarding our employees~~ **in the past and we expect such attacks will continue in the future**. Furthermore, while we have implemented data privacy and security measures that are designed to comply with applicable laws and regulations relating to privacy and data protection, some health- related and other personal information or confidential information may be transmitted to us or processed by third parties, who may not implement adequate security and privacy measures, and it is possible that laws, rules and regulations relating to privacy, data protection, or information security may be interpreted and applied in a manner that is inconsistent with our practices or those of third parties who transmit health- related and other personal information or confidential information to us or process such information on our behalf. While we may be entitled to damages if our third- party service providers fail to satisfy their privacy or security- related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. We take steps designed to detect, mitigate, and remediate vulnerabilities in our information systems (such as our hardware and / or software, including that of third parties ~~upon which~~ **with whom** we ~~rely~~ **work**). We may not, however, detect and remediate all such vulnerabilities including on a timely basis. Further, we may experience delays in deploying remedial measures and patches designed to address identified vulnerabilities. Vulnerabilities could be exploited and result in a security incident. We ~~have in the past and~~ **may in the future** expend significant resources or modify our business activities to try to protect against security incidents. Additionally, certain data privacy and security obligations may require us to implement and maintain specific security measures or industry- standard or reasonable security measures to protect our information technology systems and sensitive data. To the extent that we or third parties ~~upon which~~ **with whom** we ~~rely~~ **work** are found to have violated data security laws, rules or regulations or if we (or a third party ~~upon with~~ **whom** we ~~rely~~ **work**) experience a security incident or are perceived to have experienced a security incident, including an incident that results in a loss of, or damage to, our or our third- party vendors', collaborators', ~~or other contractors' or consultants'~~ **or other third parties with whom we work** data or ~~applications systems~~, or inappropriate disclosure of confidential or proprietary information, we could experience **material** adverse consequences including but not limited to litigation exposure, penalties and fines, regulatory actions or investigations, restrictions on processing sensitive data (including clinical trial data), reputational harm; monetary fund diversions, diversion of management attention, our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed. Any of the above could have a material adverse effect on our business, financial condition, results of operations or prospects. Our contracts may not contain limitations of liability, and even

where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims. In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive data about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Risks Related to the Ownership of Our Common Stock An active trading market for our common stock may not be sustained. Our shares of common stock began trading on the Nasdaq Global Select Market on February 5, 2021. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of our stockholders to sell their shares. The market price of our common stock may be volatile. Our stock price is, and is likely to continue to be, volatile. For example, our stock traded within a range of a high price of \$ 63.62 and a low price of \$ 10.62-64 per share for the period of February 5, 2021, our first day of trading on the Nasdaq Global Select Market, through March 13, 2024-2025. As a result of volatility, our stockholders may not be able to sell their common stock at or above the prices at which they purchased their shares. Some of the factors that may cause the market price of our common stock to fluctuate include:

- the success of existing or new competitive product candidates or technologies;
- the timing and results of preclinical studies and clinical trials for our product candidates;
- failure or discontinuation of any of our product development and research programs;
- results of preclinical studies, clinical trials or regulatory approvals of product candidates of our competitors, or announcements about new research programs or product candidates of our competitors;
- developments or changing views regarding the use of genetic medicines, including those that involve genome engineering;
- commencement or termination of collaborations for our product development and research programs;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our research programs, product candidates or clinical development programs;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or other stockholders;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- global or regional public health emergencies, and political instability, including terrorist attacks, civil unrest and actual or threatened armed conflict, such as the Russia- Ukraine and Israel- Hamas wars;
- general economic, industry and market conditions, including ~~heightened~~ **heightened** interest rates and inflation; and
- the other factors described in this “ Risk Factors ” section.

In recent years, the stock market in general, and the market for pharmaceutical and biotechnology companies in particular, has experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of the companies whose stock is experiencing those price and volume fluctuations. Broad market and industry factors may seriously affect the market price of our common stock, regardless of our actual operating performance. Following periods of such volatility in the market price of a company’ s securities, securities class action litigation has often been brought against that company. Because of the potential volatility of our stock price, we may become the target of securities litigation in the future, which could result in substantial costs and divert management’ s attention and resources from our business. If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline. The trading market for our common stock will be influenced by the research and reports that industry or financial analysts publish about us or our business. If one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline. A significant portion of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well. Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. Our outstanding shares of common stock may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act of 1933, as amended (the “ Securities Act ”), or to the extent such shares have already been registered under the Securities Act and are held by non-affiliates. Moreover, holders of a substantial number of shares of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. For example, in December 2022 we filed a registration statement on Form S- 3 to register the resale of up to 11, 627, 907 shares of common stock held by RA Capital Healthcare Fund L. P. which were purchased from us in a private placement, **and in January 2025 we filed a registration statement on Form S- 3 to register the resale of up to an aggregate of 125, 710, 335 shares of common stock held by Reprogrammed Interchange LLC and entities affiliated with RA Capital Management, L. P., consisting of (i) 55, 871, 260 shares of common stock and (ii) 69, 839, 075 shares of common stock issuable upon the exercise of outstanding warrants to purchase shares of common stock, which were purchased from us in a private placement.** We have also registered all shares of common stock that we may issue under our equity compensation plans or that are issuable upon exercise of outstanding options on a registration statement on Form S- 8 and will continue to register any additional shares that become available under such plans due to any annual, automatic increases under the terms of those plans. These shares can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates. If any of these additional shares are sold, or if it is perceived that they will be sold, in

the public market, the market price of our common stock could decline. Insiders have substantial control over our company, which could limit the ability of our other stockholders to affect the outcome of key transactions, including a change of control. Our executive officers and directors, combined with our stockholders who own more than 5 % of our outstanding common stock, and their affiliates, in the aggregate, beneficially own shares representing a substantial amount of our outstanding common stock. **In particular, our two largest stockholders, RA Capital Management, L. P. and Reprogrammed Interchange LLC, together with their affiliates, collectively hold approximately 63.0 % of our outstanding common stock, as well as warrants to purchase shares of our common stock warrants, which, if exercised, would result in such stockholders owning an even larger percentage of our outstanding common stock.** As a result, these stockholders, if they act together, may be able to influence our management and affairs and **would control** all matters requiring stockholder approval, including the election of directors and approval of significant corporate transactions, such as a merger or other sale of our company or its assets. This concentration of ownership may have the effect of delaying or preventing a change in control of our company or discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control, even if that change in control would benefit our other stockholders. This significant concentration of ownership may also adversely affect the trading price for our common stock because investors often perceive disadvantages in owning stock in companies with controlling stockholders. If we fail to establish and maintain proper and effective internal control over financial reporting, our operating results and our ability to operate our business could be harmed. Ensuring that we have adequate internal financial and accounting controls and procedures in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that needs to be re-evaluated frequently. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with generally accepted accounting principles. Implementing any appropriate changes to our internal controls may distract our officers and employees, entail substantial costs to modify our existing processes and take significant time to complete. These changes may not, however, be effective in maintaining the adequacy of our internal controls, and any failure to maintain that adequacy, or consequent inability to produce accurate financial statements on a timely basis, could increase our operating costs and harm our business. In addition, investors' perceptions that our internal controls are inadequate or that we are unable to produce accurate financial statements on a timely basis may harm our common share price. We are an "emerging growth company" and a "smaller reporting company," and the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors. We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"). We will be an emerging growth company during this year and may remain an emerging growth company until 2026. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002 ("SOX"), 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, exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved, and being permitted to provide only two years of audited financial statements. As a result, the information we provide stockholders will be different than the information that is available with respect to other public companies. For example, we did not include all of the executive compensation related information in this Annual Report that would be required if we were not an emerging growth company. We cannot predict whether investors will find our common stock less attractive if we 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. In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have availed ourselves of this extended transition period and we cannot predict whether investors will find our common stock less attractive due to this election. We are also a "smaller reporting company" and we may continue to be a smaller reporting company if either (i) the market value of our stock held by non-affiliates is less than \$ 250 million or (ii) our annual revenue is less than \$ 100 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than \$ 700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation. We will continue to incur 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 and corporate governance practices. As a public company, and particularly after we are no longer an "emerging growth company," we will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Select Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We expect that we will need to continue to hire additional accounting, finance and other personnel in connection with our efforts to comply with the requirements of being a public company, and our management and other personnel will need to continue to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements have increased and will continue to increase our legal and

financial compliance costs and make some activities more time-consuming and costly. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices. Pursuant to SOX Section 404, we are required to furnish a report by our management on our internal control over financial reporting, but while we remain an emerging growth or a smaller reporting company with less than \$ 100 million in annual revenue, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To maintain compliance with SOX Section 404 and achieve compliance within the prescribed period for the attestation report by our independent registered public accounting firm, we have and will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants, adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by SOX Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements. Our management team has broad discretion in the use of our cash reserves and may not use them effectively. Our management has broad discretion to use our cash reserves and could use our cash reserves in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could harm our business, financial condition, results of operations and prospects. Pending their use, we may invest our cash reserves in a manner that does not produce income or that loses value. We do not expect to pay any dividends for the foreseeable future. Accordingly our stockholders must rely on capital appreciation, if any, for any return on their investment. We have never declared or paid any cash dividends on our equity securities. We do not anticipate that we will pay any dividends to holders of our common stock in the foreseeable future. Instead, we plan to retain any earnings to maintain and expand our existing operations. In addition, any future credit facility that we enter into may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain for the foreseeable future. Unfavorable global economic conditions, **new tariffs** or bank closures could adversely affect our business, financial condition or results of operations. Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets, including as a result of heightened inflation and interest rates. A severe or prolonged economic downturn, or additional global financial crises, including related to potential future pandemics or the Russia- Ukraine and Israel-Hamas armed conflicts, could result in a variety of risks to our business, including weakened demand for our product candidates, if approved, or our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business. **Significant political, trade, or regulatory developments in the jurisdictions in which we sell our products, such as those stemming from the change in U. S. federal administration, are difficult to predict and may have a material adverse effect on us. Similarly, changes in U. S. federal policy that affect the geopolitical landscape could give rise to circumstances outside our control that could have negative impacts on our business operations. We could also be affected by new and increased tariffs between the United States and other countries. For example, on February 1, 2025, President Trump announced new tariffs on imports from Canada and Mexico, which were subsequently suspended for a period of one month, and a 10 % additional on imports from China. These additional tariffs and any retaliatory tariffs by other countries, could substantially increase our costs associated with the manufacture and supply of our product candidates. For example, the process by which we manufacture trem-cel and distribute it to clinical trial sites involves a third party in Canada. The global trade environment is rapidly evolving, and the United States and other countries may impose additional new tariffs, the scope of which we are unable to predict but that may adversely impact our business. If our or the activities of our third party suppliers or service providers fall within the scope of any of these or other tariffs, our costs may increase significantly. Political tensions as a result of trade policies could reduce trade volume, investment, technological exchange and other economic activities between major international economies, resulting in a material adverse effect on global economic conditions and the stability of global financial markets. Any changes in political, trade, regulatory, and economic conditions, including U. S. trade policies, could have a material adverse effect on our financial condition or results of operations.** In addition, our available cash and cash equivalents are held in accounts managed by third party financial institutions and consist of cash in our operating accounts and cash invested in money market funds. At any point in time, the funds in our operating accounts may exceed the Federal Deposit Insurance Corporation insurance limits. While we monitor the cash balances in our operating accounts and adjust the cash balances as appropriate, these cash balances could be impacted if the underlying financial institutions fail. We can provide no assurances that access to our operating cash or invested cash and cash equivalents will not be impacted by adverse conditions in the financial markets. Provisions in our certificate of incorporation and bylaws and under Delaware law could make a change in control of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management. Provisions in our amended and restated certificate of incorporation and our amended and restated bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our

common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions: • establish a classified board of directors such that not all members of the board are elected at one time; • allow the authorized number of our directors to be changed only by resolution of our board of directors; • limit the manner in which stockholders can remove directors from the board; • establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors; • require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent; • limit who may call stockholder meetings; • authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called “poison pill,” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and • require the approval of the holders of at least 66 2/3 % of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws. Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law (the “DGCL”), which prohibits a person who owns in excess of 15 % of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15 % of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. We have not elected to opt out of DGCL Section 203. These provisions could discourage potential acquisition proposals and could delay or prevent a change in control transaction. They could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in our stockholders’ best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our common stock. Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders’ ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees. Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: • any derivative action or proceeding brought on our behalf; • any action asserting a breach of fiduciary duty; • any action asserting a claim against us arising under the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws; and • any action asserting a claim against us that is governed by the internal-affairs doctrine. This provision would not apply to suits brought to enforce a duty or liability created by the Securities Exchange Act of 1934, as amended. 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 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 additional costs associated with resolving the dispute in other jurisdictions, which could seriously harm our business.