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

FORM 10-K

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the Fiscal Year Ended December 31, 2021

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the Transition Period from _____ to _____.

Commission File No. 001-38191

MUSTANG BIO, INC.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**47-3828760**
(State or Other Jurisdiction of Incorporation or Organization)	**(I.R.S. Employer Identification No.)**

377 Plantation Street
Worcester, Massachusetts 01605
(Address including zip code of principal executive offices)

(781) 652-4500
(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.0001 per share	MBIO	NASDAQ Global Market

Securities registered pursuant to section 12(g) of the Act: None.

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☒

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

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

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

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
Emerging growth company	☒		

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

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

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

Class of Common Stock	**Outstanding Shares as of March 18, 2022**
Class A Common Stock, $0.0001 par value	845,385
Common Stock, $0.0001 par value	99,445,369

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's Proxy Statement for its 2022 Annual Meeting of Stockholders are incorporated by reference into Part III hereof.

MUSTANG BIO, INC.
ANNUAL REPORT ON FORM 10-K
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SPECIAL CAUTIONARY NOTICE REGARDING FORWARD-LOOKING STATEMENTS

Certain matters discussed in this annual report on Form 10-K ("Form 10-K") may constitute forward-looking statements for purposes of the Securities Act of 1933, as amended (the "Securities Act") and the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from the future results, performance or achievements expressed or implied by such forward-looking statements. The words "anticipate," "believe," "estimate," "may," "expect" and similar expressions are generally intended to identify forward-looking statements. Our actual results may differ materially from the results anticipated in these forward-looking statements due to a variety of factors, including, without limitation, those discussed under the captions "Risk Factors," and elsewhere in this Form 10-K. All written or oral forward-looking statements attributable to us are expressly qualified in their entirety by these cautionary statements. Such forward-looking statements include, but are not limited to, statements about our:

- expectations for increases or decreases in expenses;
- expectations for the clinical and pre-clinical development, manufacturing, regulatory approval, and commercialization of our pharmaceutical product candidates or any other products we may acquire or in-license;
- use of clinical research centers and other contractors;
- expectations for incurring capital expenditures to expand our research and development and manufacturing capabilities;
- expectations for generating revenue or becoming profitable on a sustained basis;
- expectations or ability to enter into marketing and other partnership agreements;
- expectations or ability to enter into product acquisition and in-licensing transactions;
- expectations or ability to build our own commercial infrastructure to manufacture, market and sell our drug candidates;
- expectations for the acceptance of our products by doctors, patients or payors;
- ability to compete against other companies and research institutions;
- ability to secure adequate protection for our intellectual property;
- ability to attract and retain key personnel;
- ability to obtain reimbursement for our products;
- estimates of the sufficiency of our existing cash and cash equivalents and investments to finance our operating requirements, including expectations regarding the value and liquidity of our investments;
- stock price and the volatility of the equity markets;
- expected losses; and
- expectations for future capital requirements.

The forward-looking statements contained in this Form 10-K reflect our views and assumptions as of the effective date of this Form 10-K. Except as required by law, we assume no responsibility for updating any forward-looking statements.

We qualify all of our forward-looking statements by these cautionary statements.

SUMMARY RISK FACTORS

Our business is subject to risks of which you should be aware before making an investment decision. The risks described below are a summary of the principal risks associated with an investment in us and are not the only risks we face. You should carefully consider these risk factors, the risk factors described in Item 1A, and the other reports and documents that we have filed with the Securities and Exchange Commission ("SEC").

Risks Related to our Finances and Capital Requirements

- We have incurred significant losses since our inception and anticipate that we will incur continued losses for the foreseeable future. We have not generated any revenue from our development stage products, and we do not know when, or if, we will generate any revenue.
- Our short operating history makes it difficult to evaluate our business and prospects.
- Our success is contingent upon raising additional capital, which efforts may fail. Even if successful, our future capital raising activities may dilute our current stockholders, restrict our operations, cause us to relinquish proprietary rights.

Risks Pertaining to our Business Strategy, Structure and Organization

- Our future growth and success depend on our ability to successfully develop and commercialize our product candidates, which we have yet to do.
- Our growth and success depend on our acquiring or in-licensing products or product candidates and integrating such products into our business, and we may have limited growth opportunities if we fail to do so.
- Our future success is highly dependent on the successful development of our CAR T technology and product candidates.

Risks Inherent in Drug Development and Commercialization

- Preclinical development is highly speculative and carries a high failure risk.
- We may not receive the required regulatory approvals for any of our product candidates on our projected timelines, if at all, which may result in increased costs and delay our ability to generate revenue.
- We may not obtain the desired labeling claims or intended uses for product promotion, or favorable scheduling classifications, to successfully promote our products.
- If a product candidate demonstrates adverse side effects, we may need to abandon or limit the development of such product candidate.
- Even if a product candidate is approved, it may be subject to various post-marketing requirements, including studies or clinical trials, and increased regulatory scrutiny.
- Our competitors may develop treatments for our or our partner companies' products' target indications, which could limit our product candidates' commercial opportunity and profitability.
- If our products are not broadly accepted by the healthcare community, the revenues from any such product will likely be limited.
- Any successful products liability claim related to any of our current or future product candidates may cause us to incur substantial liability and limit the commercialization of such products.
- Our gene therapy product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval.

Risks Related to Reliance on Third Parties

- We rely, and expect to continue to rely, on third parties to conduct our preclinical studies and clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials or complying with applicable regulatory requirements.
- We contract with third parties for the manufacture of our product candidates for preclinical and clinical testing and may also do so for commercialization, if and when our product candidates are approved.
- We rely on clinical data and results obtained by third parties, which may prove inaccurate or unreliable.
- We may need to license certain intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

Risks Relating to Legislation and Regulation Affecting the Biopharmaceutical and Other Industries

- We operate in a heavily regulated industry, and we cannot predict the impact that any future legislation or administrative or executive action may have on our operations.
- We may be subject to anti-kickback, fraud and abuse, false claims, transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.
- We are subject to numerous environmental, health and safety laws and regulations and could become subject to fines or penalties or incur costs that could harm our business

Risks Pertaining to Intellectual Property and Potential Disputes with Licensors Thereof

- If we are unable to maintain sufficient patent protection for our technology and products, our competitors could develop and commercialize products similar or identical to ours and our ability to successfully commercialize our technology and products could be impaired.
- We depend on our licensors for the maintenance and enforcement of to maintain and enforce the intellectual property covering certain of our product candidates.
- We or our licensors may be subject to costly and time-consuming litigation for infringement of third-party intellectual property rights or to enforce our or our licensors' patents.
- Any dispute with our licensors may affect our ability to develop or commercialize our product candidates.

Risks Relating to Our Control by Fortress Biotech, Inc. ("Fortress")

- Fortress controls a voting majority of our common stock and has the right to receive significant share grants annually, which will result in dilution of our other stockholders and could reduce the value of our common stock.
- We have entered into certain agreements with Fortress and may have received better terms from unaffiliated third parties.

Risks Related to Conflicts of Interest

- We share certain directors with Fortress, which could create conflicts of interest between us and Fortress.

PART I

Item 1. Business

OVERVIEW

Mustang Bio, Inc. ("Mustang," "We," "Us" or the "Company") is a clinical-stage biopharmaceutical company focused on translating today's medical breakthroughs in cell and gene therapies into potential cures for hematologic cancers, solid tumors and rare genetic diseases. We aim to acquire rights to these technologies by licensing or otherwise acquiring an ownership interest in the technologies, funding their research and development and eventually either out-licensing or bringing the technologies to market.

Our pipeline is currently focused in three core areas: gene therapies for rare genetic disorders, chimeric antigen receptor ("CAR") engineered T cell ("CAR T") therapies for hematologic malignancies and CAR T therapies for solid tumors. For each therapy we have partnered with world class research institutions. For our gene therapies, we have partnered with St. Jude Children's Research Hospital ("St. Jude") in the development of a first-in-class *ex vivo* lentiviral treatment of X-linked severe combined immunodeficiency ("XSCID") and with Leiden University Medical Centre ("LUMC") for RAG1 severe combined immunodeficiency ("RAG1-SCID"). For our CAR T therapies we have partnered with the City of Hope National Medical Center ("COH"), Fred Hutchinson Cancer Research Center ("Fred Hutch"), Nationwide Children's Hospital ("Nationwide") and the Mayo Foundation for Medical Education and Research ("Mayo Clinic").

Gene Therapies

In partnership with St. Jude, our XSCID gene therapy programs (MB-107 and MB-207) are being conducted under an exclusive license to develop a potentially curative treatment for XSCID, a rare genetic immune system condition in which affected patients do not live beyond infancy without treatment. This first-in-class *ex vivo* lentiviral gene therapy is currently in two Phase 1/2 clinical trials involving two different autologous cell products: a multicenter trial of the MB-107 product in newly diagnosed infants sponsored by St. Jude and a single-center

trial of the MB-207 product in previously transplanted patients sponsored by the National Institutes of Health ("NIH"). In January 2021 we received approval to proceed with our Investigational New Drug ("IND") application with the U.S. Food and Drug Administration ("FDA") to initiate a pivotal non-randomized multicenter Phase 2 clinical trial of MB-107 in newly diagnosed infants with XSCID who are under the age of two. In January 2022, the FDA issued a hold, pending Chemistry, Manufacturing and Controls ("CMC") clearance, on our IND application to conduct a pivotal non-randomized multicenter Phase 2 clinical trial of MB-207 in previously transplanted XSCID patients.

CAR T Therapies

Our pipeline of CAR T therapies is being developed under exclusive licenses from several world class research institutions. Our strategy is to license these technologies, support preclinical and clinical research activities by our partners and transfer the underlying technology to our cell processing facility located in Worcester, Massachusetts, in order to conduct our own clinical trials.

We are developing CAR T therapies for hematologic malignancies in partnership with COH targeting CD123 (MB-102) and CS1 (MB-104) and with Fred Hutch targeting CD20 (MB-106). Phase 1 clinical trials sponsored by COH for MB-102 and MB-104 and by Fred Hutch for MB-106 are underway. In the third quarter of 2019 the FDA approved our IND application to initiate a multi-center Phase 1/2 clinical trial of MB-102, and our clinical trial began enrollment in 2020 for the treatment of patients with blastic plasmacytoid dendritic cell neoplasm. In May 2021, the FDA approved our IND application to initiate a multi-center Phase 1/2 clinical trial of MB-106, and we expect to begin the treatment of patients with non-Hodgkin lymphoma and chronic lymphocytic leukemia in the first half of 2022. We plan to file an IND for a multicenter Phase 1/2 trial for MB-104 for the treatment of patients with multiple myeloma once COH has established a safe and effective dose.

We are also developing CAR T therapies for solid tumors in partnership with COH targeting IL13Rα2 (MB-101), HER2 (MB-103) and PSCA (MB-105). In addition, we have partnered with Nationwide for the C134 oncolytic virus (MB-108) in order to enhance the activity of MB-101 for the treatment of patients with glioblastoma multiforme ("GBM"). Phase 1 clinical trials sponsored by COH for MB-101, MB-103 and MB-105 are underway. A Phase 1 clinical trial sponsored by the University of Alabama at Birmingham ("UAB") for MB-108 began during the third quarter of 2019 and, in the second half of 2022, we plan to file an IND for the combination of MB-101 and MB-108 – which is referred to as MB-109 – for the treatment of patients with relapsed or refractory GBM and anaplastic astrocytoma,. In the third quarter of 2019, we announced that COH had started enrolling patients on a Phase 1 clinical trial of MB-101 in combination with nivolumab (commercial name: Opdivo®) and ipilimumab (commercial name: Yervoy®) in patients with recurrent malignant glioma (ClinicalTrials.gov Identifier: NCT04003649). In the fourth quarter of 2020 we announced that COH had initiated a Phase 1, two-arm clinical trial of MB-101 in patients with leptomeningeal brain tumors (e.g., glioblastoma, ependymoma or medulloblastoma; ClinicalTrials.gov Identifier: NCT04003649).

We also plan to file INDs and initiate our own clinical trials for MB-103 for the treatment of patients with metastatic breast cancer to brain and for MB-105 for the treatment of patients with prostate and pancreatic cancer. Finally, the Company is collaborating with the Mayo Clinic to develop a novel technology that may be able to transform the administration of CAR T therapies and potentially be used as an off-the-shelf therapy. Mustang plans to file an IND application for a multicenter Phase 1 clinical trial once a lead construct has been identified.

To date, we have not received approval for the sale of our product candidates in any market and, therefore, have not generated any product sales from our product candidates. In addition, we have incurred substantial operating losses since our inception, and expect to continue to incur significant operating losses for the foreseeable future and may never become profitable. As of December 31, 2021, we have an accumulated deficit of $251.8 million.

We are a majority-controlled subsidiary of Fortress Biotech, Inc. ("Fortress").

CORPORATE INFORMATION

Mustang Bio, Inc. was incorporated in Delaware on March 13, 2015. Our executive offices are located at 377 Plantation Street, Worcester, Massachusetts 01605. Our telephone number is (781) 652-4500, and our email address is info@mustangbio.com.

Our website address is www.mustangbio.com. The information set forth on our website is not a part of this report. We will make available free of charge through our website our annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, and any amendments to these reports, as soon as reasonably practicable after we electronically file such material with, or furnish such material to, the SEC. We are not including the information on our website as a part of, nor incorporating it by reference into, this report. The SEC maintains a website that contains annual, quarterly, and current reports, proxy and information statements, and other information that issuers (including us) file electronically with the SEC. The SEC's website address is https://www.sec.gov/.

PRODUCTS UNDER DEVELOPMENT

Gene Therapies for Rare Genetic Disorders

MB-107 and MB-207 (Ex vivo Lentiviral Therapy for X-linked Severe Combined Immunodeficiency (XSCID))

XSCID is a rare genetic immune system condition also known as bubble boy disease, in which affected patients do not live beyond infancy without treatment. Mustang Bio's first-in-class *ex vivo* lentiviral gene therapy for XSCID is currently being administered as two distinct cellular products using the same lentiviral vector in two phase 1/2 clinical trials: (1) a multicenter trial of MB-107 in newly diagnosed patients being led by St. Jude and including also UCSF Benioff Children's Hospital San Francisco ("UCSF") and Seattle Children's Hospital ("Seattle Children's") (ClinicalTrials.gov Identifier: NCT01512888) and (2) a single center trial of MB-207 at the NIH in patients who have previously undergone hematopoietic stem cell transplantation (ClinicalTrials.gov Identifier: NCT01306019).

The most recent peer-reviewed presentations by the respective investigators for these two trials occurred at the 61st Annual Meeting of the American Society of Hematology ("ASH") in December 2019, at which time 24 patients had been treated in total. Eleven patients under the age of two years had been treated at St. Jude and UCSF and thirteen patients 3 to 34 years of age had been treated at the NIH.

The existing data from these 24 patients were encouraging. In the initial stage of accrual to the Phase 1/2 NIH trial, eight patients (referred to as Cohort A) were followed for 3 to 7 years. Among Cohort A, seven patients aged 3 to 23 years increased host T cells chimerism from 0-2% to 28-93% and had normal T cell proliferation response. These seven patients also normalized their IgM levels, and four of these patients were able to discontinue immunoglobulin replacement therapy. In addition, gradual clinical benefit was observed in the clearance of chronic norovirus and associated abdominal complaints, malabsorption, and growth retardation, with six of seven affected patients being cured of their disease. Five of six patients resolved their protein-losing enteropathy.

While the Cohort A results were impressive, the relatively inefficient transduction of hematopoietic stem/progenitor cells ("HSPCs") required large quantities of vector. This resulted in relatively low vector copy number in myeloid cells in some patients, with delayed immune cell recovery and persistent clinical disease, especially in the last patient treated (patient 8). To address this, NIH developed a refined enhanced transduction ("ET") procedure and incorporated two transduction enhancers: LentiBOOST™ 1mg/mL and dimethyl prostaglandin 2 (dmPGE2; 1μM).

In addition to the Cohort A results, the NIH presentation at the 2019 ASH Annual Meeting included data from six ET patients (referred to as Cohort B) treated from February to June 2019, including re-treatment of patient 8. Prior to undergoing gene therapy, the patients, who were aged 12 to 36 years, had significant problems with donor T cell infiltration of liver, bone marrow and kidneys and had nearly absent B and NK cells. The ET procedure achieved much greater transduction efficiencies than were observed in Cohort A, with greater than 10-fold less vector, and resulted in faster immune reconstitution and more significant clinical benefit by 3 months. As noted by the investigators, longer follow-up will be required to know if the increased vector marking using the ET regimen will prove to be stable and safe long term.

In all NIH patients, the low-dose, nonmyeloablative busulfan pretreatment conditioning was well tolerated, and of a low enough intensity to avoid the need for transfusions of red blood cells or platelets. No evidence of malignant transformation was observed.

Subsequent to the initiation of the NIH trial, eleven patients under two years old who had not previously undergone hematopoietic stem cell transplantation ("HSCT") were treated with the *ex-vivo* gene therapy in a St. Jude/UCSF/Seattle Children's Phase 1/2 trial, resulting in highly encouraging results. Low-dose busulfan conditioning caused non-hematologic adverse events in only two patients (mild mucositis; mucositis, hair loss), and no patients required blood product support. All 11 patients had robust hematopoietic recovery within 3-4 weeks post cell infusion, and the nine patients who had a follow-up of greater than 3 months achieved normal-for-age T-cell and natural killer ("NK")-cell numbers within 3-4 months post gene therapy. Five patients were reported to have successfully discontinued intravenous immunoglobulin ("IVIG") therapy, of whom 3 responded to vaccines. Median vector copy number ("VCN") at 12 months post-gene therapy in the seven patients who had a follow-up of greater than 12 months was 2.25 VCN/cell (range: 1.24-3.03) in T cells, 0.34 VCN/cell (range: 0.23-1.25) in B cells, 1.55 VCN/cell (range 1.27-3.39) in NK cells, and 0.08 VCN/cell (range: 0.03-0.76) in myeloid cells in peripheral blood, and 0.10 (range: 0.05-0.66) in CD34+ bone marrow cells, respectively. With a median follow-up of 23.6 months, no evidence of malignant transformation was observed.

In a press release dated February 2, 2021, Mustang Bio provided a high-level update to the two ongoing phase 1/2 trials. That press release disclosed that all 11 patients enrolled on the St. Jude/USCF/Seattle Children's trial continued to do well, and 5 additional patients had been enrolled at the time of the most recent analysis in early September 2020. At that time, follow-up for these 16 patients ranged from 3 months to 47 months. Similar to previous reports, the therapy continued to be well tolerated in all patients, and stable vector marking was noted in

all lineages, with successful engraftment of genetically-modified T-, B-, & NK-cells. All patients cleared pre-existing infections, no new severe infections were noted, and all patients were outpatients. Finally, there was no evidence of malignant transformation at a median follow up of 2 years.

The February 2, 2021, press release further disclosed that, of the 6 Cohort A patients who were alive at the time of the 2019 NIH data readout and who did not undergo repeat therapy, 3 patients were able to discontinue chronic intravenous immunoglobulin (IVIG) and experienced sustained restoration of humoral responses to immunization. The remaining 3 patients had reduced IVIG requirements. All chronic norovirus infections were resolved, and the quality of life of all patients had improved significantly. The original 6 patients in Cohort B also continued to do well, with the longest follow-up being 22 months. Two additional patients were successfully treated with transduction enhancers, for a total of 8 patients in Cohort B. As was the case in Cohort A, no serious adverse events related to treatment were reported other than hematologic related to low-dose busulfan conditioning, and there was no evidence of malignant transformation.

MB-110 (Ex vivo Lentiviral Therapy for RAG1 Severe Combined Immunodeficiency (SCID))

In partnership with LUMC, our RAG1-SCID gene therapy program (MB-110) is being conducted under an exclusive license to develop a first-in-class *ex vivo* treatment for a rare genetic immune system condition. Severe combined immunodeficiency due to complete recombinase-activating gene-1 (RAG1) deficiency is a rare, genetic severe combined immunodeficiency disorder due to null mutations in the RAG1 gene resulting in less than 1% of wild type V(D)J recombination activity. Patients present with neonatal onset of life-threatening, severe, recurrent infections by opportunistic fungal, viral and bacterial micro-organisms, as well as skin rashes, chronic diarrhea, failure to thrive and fever. Immunologic observations include profound T- and B-cell lymphopenia, low or absent serum immunoglobulins, and normal natural killer cell counts. As is the case with other types of SCID, RAG1-SCID is fatal in infancy unless immune reconstitution is achieved with hematopoietic stem cell transplantation (HSCT).

MB-110, which includes low-dose conditioning prior to reinfusion of the patients' own gene-modified blood stem cells, is currently being evaluated in a Phase 1/2 multicenter clinical trial in Europe. The ongoing clinical trial has enrolled its first patient, and additional clinical sites are expected to be added in the near future. The RAG1-SCID program has been granted Orphan Drug Designation by the European Medicines Agency.

Mustang also established an ongoing partnership with Frank J. Staal, Ph.D., professor of Molecular Stem Cell Biology and molecular immunologist at LUMC, whose laboratory developed the therapy. Dr. Staal will continue the development of additional lentiviral gene therapies in his lab, to which Mustang Bio has rights under the agreement.

CAR T Therapies for Hematologic Malignancies

MB-102 (CD123 CAR T cell Program for BPDCN, AML and high-risk MDS)

CD123 is a subunit of the heterodimeric interleukin-3-receptor ("IL-3R") which is widely expressed on human hematologic malignancies including blastic plasmacytoid dendritic cell neoplasm ("BPDCN") and acute myeloid leukemia ("AML"). In addition, CD123 can be found on the surface of B cell acute lymphoblastic leukemia ("B-ALL"), hairy cell leukemia, myelodysplastic syndrome ("MDS"), chronic myeloid leukemia ("CML") and Hodgkin lymphoma.

Of these malignancies, we are currently investigating CD123 as a target for adoptive cellular immunotherapy in BPDCN, since high CD123 expression is associated with enhanced cell proliferation, increased resistance of these cells to apoptosis, and poor clinical prognosis. Depending on the early results in this patient population, we may broaden the inclusion criteria to include AML and high-risk MDS ("hrMDS"). CD123 is overexpressed in the vast majority of cases of AML and hrMDS and in essentially all cases of BPDCN.

Acute myeloid leukemia is a cancer of the myeloid line of blood cells characterized by rapid growth of abnormal white blood cells that accumulate in the bone marrow. AML is the most common form of acute leukemia. Although AML is a relatively rare disease, there are approximately 20,000 new cases per year in the U.S. and 10,000 deaths per year, accounting for approximately 1.8% of cancer deaths in the U.S. [Source: The Surveillance, Epidemiology, and End Results ("SEER") Program of the National Cancer Institute]. AML standard of care involves chemotherapy to induce remission followed by additional chemotherapy or hematopoietic stem cell transplant. Allogeneic stem cell transplantation is the preferred treatment for AML following a second remission. It can lead to a 5-year disease-free survival in 26% of patients. Unfortunately, however, currently only about half of relapsed patients are able to achieve a second remission with traditional chemotherapy agents. Patients who do not achieve a second remission are much less likely to benefit from transplantation and face a dismal outcome.

MDS is a heterogeneous group of malignant hematopoietic stem cell disorders characterized by dysplastic and ineffective blood cell production and a variable risk of transformation to acute leukemia. Patients with MDS have varying reductions in the production of red blood cells, platelets, and mature granulocytes that may also exhibit functional defects; these abnormalities often result in anemia, bleeding, and increased risk of infection. The precise incidence of de novo MDS is not known; conservative estimates from cancer databases suggest that there are approximately 10,000 cases diagnosed annually in the U.S. The actual incidence of MDS is likely higher than that predicted by cancer databases, since the nonspecific symptoms may evade detection in early stages of the disease and suspected cases may not undergo definitive testing (i.e., bone marrow biopsy) due to comorbidities. Investigations that have analyzed reimbursement claims have estimated the incidence in the U.S. to be 30,000 to 40,000 new cases per year. MDS occurs most commonly in older adults, with a median age at diagnosis in most series of ≥65 years and a male predominance.

MDS and AML lie along a disease continuum, with distinction between the two largely made based upon the percentage of myeloblasts, which are immature cells with large nuclei, nucleoli, and a scant rim of dark blue cytoplasm, suggesting an underlying malignant hematologic disorder. In the current World Health Organization ("WHO") classification system, blast forms must account for less than 20% of the total cells of the bone marrow aspirate and peripheral blood in order to meet the criteria for MDS.

MDS prognosis is often assessed using the revised International Prognostic Scoring System ("IPSS-R"), which takes into account cytogenetics, percentage of bone marrow blasts, and the degree of anemia, thrombocytopenia, and neutropenia. This System categorizes patients into very low, low, intermediate, high, and very high risk MDS. High risk and very high risk MDS are generally progressive in nature and can easily progress to AML. Treatment is stratified according to medical fitness in a manner similar to that for older patients with AML. Patients who are medically fit or of intermediate fitness are generally evaluated soon after diagnosis to determine their suitability for allogeneic hematopoietic cell transplantation. For patients who are not candidates for intensive treatment, care is focused on relieving symptoms and improving the quality of life and might involve lower intensity treatment, for example, with azacitidine, decitabine, or targeted therapy. Patients with recurrent or refractory higher risk MDS may be encouraged to participate in clinical trials. Outside of a clinical trial, the management of patients with recurrent or refractory MDS is largely dependent on the patient's prior therapy.

BPDCN is categorized by the WHO under AML. Most often, BPDCN presents with features of both lymphoma and leukemia. There is little data about BPDCN and the only approved drug for this disease is tagraxofusp-erzs, which is indicated for the treatment of adult and pediatric patients with both treatment-naïve and previously-treated BPDCN. The average age at diagnosis is 60 to 70 years. BPDCN is very often misdiagnosed and under-reported. The skin is the most frequently involved site of disease (80 percent of cases). However, BPDCN usually progresses with bone marrow involvement and a decrease in red blood cell, white blood cell and platelet counts. The lymph nodes and spleen may also be involved. Common misdiagnoses for BPCDN include non-Hodgkin lymphoma ("NHL"), AML, leukemia cutis [a nonspecific term used for cutaneous (skin) manifestation of any type of leukemia], melanoma (a type of skin cancer), and lupus erythematosus (chronic inflammatory disease that occurs when the body's immune system attacks its own tissues and organs). There are no data or randomized clinical trials that can define the best first treatment for patients with BPDCN. In addition to the emerging use of tagraxofusp-erzs, which was approved by the FDA in December 2018, treatment sometimes includes therapies that are used for AML, ALL, or lymphoma. The time for which a patient responds to these treatments is usually short. After a relapse, second remissions with conventional chemotherapy are difficult to achieve. Allogeneic hematopoietic stem cell transplant ("allo-SCT"), especially if offered in first remission, may result in longer remissions. The current recommendation is for BPDCN patients to be evaluated for an allo-SCT as soon as possible and to begin searching for a donor.

The use of CAR T immunotherapy in relapsed BPDCN, AML, and hrMDS patients may offer the potential to achieve a complete or longer lasting remission. We have developed CD123-targeted CAR T cells designed to be activated, to proliferate, and to kill CD123-expressing tumor cells [Mardiros A *et al*. *Blood*. 2013;122(18):3138-3148]. The therapy is designed to recognize and eliminate malignant cells, leading to remission in patients with relapsed or refractory BPDCN, AML, and hrMDS, and could serve as a bridge to potentially curative allogeneic stem cell transplant. The manufacturing process genetically modifies T cells isolated from peripheral blood mononuclear cells in order to express a CD123-specific, hinge-optimized, CD28 co-stimulatory domain-expressing CAR.

In October 2020, we announced the dosing of the first patient in a multicenter Phase 1/2 clinical trial of MB-102 in patients with relapsed or refractory BPDCN (Clinicaltrials.gov Identifier: NCT04109482). This is also the first clinical trial under a Mustang IND in which a patient was dosed with cells processed in our manufacturing facility.

MB-104 (CS1 CAR T for Multiple Myeloma and Light Chain Amyloidosis)

CS1 (also known as CD319, CRACC and SLAMF7) was identified as an NK cell receptor regulating immune functions. It is also expressed on B cells, T cells, dendritic cells, NK-T cells, and monocytes. CS1 is overexpressed in multiple myeloma ("MM") and light chain amyloidosis ("AL"), which makes it a good target for immunotherapy. A humanized anti-CS1 antibody, elotuzumab (Empliciti™), has

shown promising results in clinical studies and was initially approved by the FDA in 2015 in combination with lenalidomide and dexamethasone for the treatment of patients with multiple myeloma who have received one to three prior therapies. Despite great advances in treatment, MM remains an incurable malignancy of plasma cells. AL is a protein deposition disorder that is a result of a plasma cell dysplasia, similar to MM. Immunotherapy is an attractive approach for AL because of the low burden of disease. Our academic partners at COH have developed a novel second generation CS1-specific CAR T cell therapy. In preclinical studies, they have demonstrated efficacy of these CAR T cells, both *in vitro* and *in vivo*, within the context of clinically relevant models of MM and AL. COH is evaluating the safety of this CS1-specific CAR T cell therapy in a Phase 1 trial that commenced in the first half of 2019 (ClinicalTrials.gov Identifier: NCT03710421). Once COH has established a safe and effective dose for MB-104 in this trial, we expect to file an IND for a multicenter Phase 1/2 trial for the treatment of patients with MM.

MB-106 (CD20 CAR T for B cell non-Hodgkin lymphoma (NHL) and chronic lymphocytic leukemia (CLL))

CD20 is a promising target for immunotherapy of B-cell malignancies. CD20 is a B-cell lineage-specific phosphoprotein that is expressed in high, homogeneous density on the surface of more than 95% of B-cell NHL and CLL. CD20 is stable on the cell surface with minimal shedding or internalization upon binding antibody and is present at only nanomolar levels as soluble antigen. It is well established as an effective immunotherapy target, with extensive studies demonstrating improved tumor responses and survival of B-NHL patients treated with rituximab and other anti-CD20 antibodies. Importantly, CD20 continues to be expressed on the lymphoma cells of most patients with relapsed B-NHL despite repetitive rituximab treatments, and loss of CD20 expression is not a major contributor to treatment resistance. Thus, there is strong rationale for testing CD20 CAR T cells as an immunotherapy for NHL.

More than 70,000 new cases of NHL are diagnosed each year in the United States, and more than 19,000 patients die of this group of diseases annually. Most forms of NHL including follicular lymphoma, mantle cell lymphoma, marginal zone lymphoma, lymphoplasmacytic lymphoma, and small lymphocytic lymphoma, which account collectively for ~45% of all cases of NHL, are incurable with available therapies, except for allogenic hematopoietic stem cell transplant ("allo-SCT"). However, many NHL patients are not suitable candidates for allo-SCT, and this treatment is also limited by significant rates of morbidity and mortality due to graft-versus-host disease. Aggressive B-cell lymphomas such as diffuse large B-cell lymphoma account for an additional 30-35% of NHL. The majority of patients with aggressive B-NHL are successfully treated with combination chemotherapy, but a significant proportion relapse or have refractory disease, and the outcome of these patients is poor. Innovative new treatments are therefore urgently needed.

Chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) is a mature B cell neoplasm characterized by a progressive accumulation of monoclonal B lymphocytes. CLL is considered to be identical (i.e., one disease with different manifestations) to the non-Hodgkin lymphoma SLL. The malignant cells seen in CLL and SLL have identical pathologic and immunophenotypic features. The term CLL is used when the disease manifests primarily in the blood, whereas the term SLL is used when involvement is primarily nodal.

CLL/SLL is the most common leukemia in adults in Western countries, accounting for approximately 25 to 35 percent of all leukemias in the United States. It is estimated that 21,250 new cases of CLL/SLL will be diagnosed in the United States in 2021. CLL/SLL is considered to be mainly a disease of older adults, with a median age at diagnosis of approximately 70 years; however, it is not unusual to make this diagnosis in younger individuals (e.g., from 30 to 39 years of age). The incidence increases rapidly with increasing age. The natural history of CLL is extremely variable, with survival times from initial diagnosis that range from approximately 2 to 20 years, and a median survival of approximately 10 years.

Most patients will have a complete or partial response to initial therapy. However, conventional therapy for CLL is not curative and most patients experience relapse. In addition, many patients will require a change in therapy due to intolerance. Patients with CLL are generally elderly with a median age older than 70 years, and due to the relatively benign course of the disease in the majority of patients, only selected patients are candidates for intensive treatments such as HCT. Innovative new treatments with a favorable safety profile are therefore urgently needed for patients with relapsed and refractory disease.

Fred Hutch has an open IND for a Phase 1/2 clinical study to evaluate the anti-tumor activity and safety of administering CD20-directed third-generation CAR T cells incorporating both 4-1BB and CD28 co-stimulatory signaling domains (MB-106) to patients with relapsed or refractory B-cell NHL or CLL (ClinicalTrials.gov Identifier: NCT03277729). Secondary endpoints include safety and toxicity, preliminary antitumor activity as measured by overall response rate and complete remission rate, progression-free survival, and overall survival. The trial will also assess CAR T cell persistence and determine the potential immunogenicity of the cells, and Mustang together with Fred Hutch will determine a recommended Phase 2 dose. Fred Hutch intends to enroll approximately 30 subjects on the trial, which is being led by principal investigator Mazyar Shadman, M.D., M.P.H., Assistant Member of Fred Hutch's Clinical Research Division. This IND was submitted on February 24, 2017, with Fred Hutch as the sponsor.

The IND was amended in 2019 to incorporate an optimized manufacturing process that had been developed in collaboration with Mustang. Due to the expected increased potency of the CAR-T cells, the dose in the first cohort treated with this optimized manufacturing process was reduced back to the cohort 1 dose of 3.3 x 10^5 CAR T cells/kg. The first patient treated in this cohort had follicular lymphoma that had relapsed after initial therapy, maintenance therapy, and two salvage regimens. She achieved a complete response ("CR") on Day 28, and no cytokine release syndrome ("CRS") or neurologic toxicity was observed. While this initial success using the optimized MB-106 process is important, additional clinical testing is necessary, and accrual to the trial continues.

In December 2020, at the 62nd American Society of Hematology Annual Meeting, Mustang and Fred Hutch announced interim data in patients with relapsed or refractory B-cell NHL from the ongoing Phase 1/2 clinical trial of MB-106 at Fred Hutch. Data presented by Fred Hutch reported on an initial seven patients treated without response and without significant CAR T cell expansion or persistence. Among these 7 patients there was one occurrence of CRS (CRS; grade 3 – unexplained alkaline phosphatase elevation in the setting of fever), and there were no occurrences of immune effector cell-associated neurotoxicity syndrome ("ICANS"). Subsequently the trial was put on hold while Fred Hutch collaborated with Mustang to undertake a major modification in the cell manufacturing process.

Following IND amendment to implement this modified cell processing, 9 patients – 7 with follicular lymphoma and 2 with mantle cell lymphoma – were treated at 4 different dose levels ranging from 1 x 10^5 CAR T cells/kg to 3.3 x 10^6 CAR T cells/kg. The overall response rate was 89% (8/9), the complete response rate was 44% (4/9), and there was good expansion and persistence of CAR T cells. All complete responses were ongoing at the time of data disclosure. One patient experienced a grade 1 episode of CRS, and no patients experienced ICANS. Mustang plans to file an IND at the end of the first quarter of 2021 to enable the initiation of a multicenter Phase 1/2 trial of MB-106.

In December 2021, we announced MB-106 data presented at ASH2021. Dr. Mazyar Shadman of Fred Hutch updated interim data showing a 95% overall response rate, 65% complete response rate and favorable safety profile from the ongoing Phase 1/2 clinical trial for NHL and CLL. No patient experienced CRS or ICANS ≥ grade 3.

CAR T Therapies for Solid Tumors

MB-101 (IL13Rα2 CAR T Cell Program for Glioblastoma)

GBM is the most common brain and central nervous system ("CNS") cancer, accounting for 45.2% of malignant primary brain and CNS tumors, 54% of all gliomas, and 16% of all primary brain and CNS tumors. More than 13,000 new glioblastoma cases were predicted in the U.S. for 2020. Malignant brain tumors are the most common cause of cancer-related deaths in adolescents and young adults aged 15-39 and the most common cancer occurring among 15-19 year-olds in the U.S. While GBM is a rare disease [2-3 cases per 100,000 persons per year in the U.S. and European Union ("EU")], it is quite lethal, with five-year survival rates historically under 10%. Standard of care therapy consists of maximal surgical resection, radiation and chemotherapy with temozolomide, which, while rarely curative, is shown to extend median overall survival from 4.5 to 15 months. GBM remains difficult to treat due to the inherent resistance of the tumor to conventional therapies.

Immunotherapy approaches targeting brain tumors offer promise over conventional treatments. IL13Rα2 is an attractive target for CAR T therapy, as it has limited expression in normal tissue but is overexpressed on the surface of greater than 50% of GBM tumors. CAR T cells are designed to express membrane-tethered IL-13 receptor ligand ("IL-13") mutated at a single site (glutamic acid at position 13 to a tyrosine; E13Y) with high affinity for IL13Rα2 and reduced binding to IL13Rα1 in order to reduce healthy tissue targeting (Kahlon KS *et al. Cancer Research.* 2004;64:9160-9166).

We are developing an optimized CAR T product incorporating enhancements in CAR T design and T cell engineering to improve antitumor potency and T cell persistence. We include a second-generation hinge-optimized CAR containing mutations in the IgG4 linker to reduce off-target Fc interactions (Jonnalagadda M *et al. Molecular Therapy.* 2015;23(4):757-768.). We also include the 4-1BB (CD137) co-stimulatory signaling domain for improved survival and maintenance of CAR T cells. Finally, we incorporate the extracellular domain of CD19 as a selection/tracking marker. In order to further improve persistence, either central memory T-cells (T_{CM}; Arms 1 – 4) or enriched CD62L+ naïve and memory T cells ($T_{N/MEM}$; Arm 5) are isolated and enriched. The manufacturing process limits *ex vivo* expansion, which is designed to reduce T cell exhaustion and maintain a T_{CM} or $T_{N/MEM}$ phenotype. These CAR modified T_{CM} and $T_{N/MEM}$ cells are shown to be more potent and persistent than earlier generations of CAR T cells, based on experiments with CAR Ts in mouse xenograft models of GBM.

Our academic partners at COH have an open IND to assess the feasibility and safety of using T_{CM} or $T_{N/MEM}$ enriched IL13Rα2-specific CAR engineered T cells for clinical study participants with recurrent/refractory malignant glioma (ClinicalTrials.gov Identifier: NCT02208362). This IND was submitted in October 2014, with COH as the sponsor. COH has enrolled and treated 65 patients as of

December 31, 2021. In the annual meeting of the American Association for Cancer Research in April 2018, our collaborators at COH presented the preliminary data for patients enrolled on Arm 2 of the protocol (the "Intracavitary Arm"). The investigators reported that the CAR T cells were well-tolerated, meaning that no dose-limiting toxicities had been seen to date. In 2016 the investigators reported on a patient that they determined had a complete response to treatment based on the imaging and clinical features set forth by the Response Assessment in Neuro-Oncology Criteria ("RANO"). This result was published as a case report in the *New England Journal of Medicine* (Brown CE et al. *NEJM*. 2016;375:2561-9). As described in the paper, a patient with recurrent multifocal glioblastoma received multiple infusions of IL13Rα2-specific CAR T cells over 220 days through two intracranial delivery routes – infusions into the resected tumor cavity followed by infusions into the ventricular system. Intracranial infusions of IL13Rα2-targeted CAR T cells were not associated with any toxic effects of grade 3 or higher. After CAR T-cell treatment, regression of all intracranial and spinal tumors was observed, along with corresponding increases in levels of cytokines and immune cells in the cerebrospinal fluid. This clinical response was sustained for 7.5 months after the initiation of CAR T-cell therapy; however, the patient's disease eventually recurred at four new locations that were distinct and non-adjacent to the original tumors, and biopsy of one of these lesions showed decreased expression of IL13Rα2. With enrollment in this Phase 1 study nearly complete, COH has established the recommended Phase 2 dose, schedule and route of administration, as well as optimal T cell selection. Results from this study have laid the foundation for 3 new MB-101 studies:

1. MB-101 with or without nivolumab and ipilimumab in treating patients with recurrent or refractory glioblastoma (currently enrolling patients; ClinicalTrials.gov Identifier: NCT04003649);

2. MB-101 in treating patients with recurrent or refractory glioblastoma with a substantial component of leptomeningeal disease (currently enrolling patients; ClinicalTrials.gov Identifier: NCT04661384);

3. MB-101 in combination with the C134 oncolytic virus (MB-108) in treating patients with recurrent or refractory glioblastoma (IND filing expected in the second half of 2022). This combination will be referred to as MB-109.

MB-103 (HER2 CAR T for GBM & Metastatic Breast Cancer to Brain)

HER2/neu ("HER2") is a growth-promoting protein on the outside of all breast cells. Breast cancer cells with higher than normal levels of HER2 are called HER2-positive ("HER2+"). These cancers tend to grow and spread faster than other breast cancers. Breast cancer is the most commonly diagnosed cancer in women, with over 42,000 women in the United States expected to die from advanced metastatic disease in 2020. Approximately 20% to 25% of breast cancers overexpress HER2, which is an established therapeutic target of both monoclonal antibodies (mAbs) and receptor tyrosine kinase inhibitors. With the advent of effective mAbs directed against HER2, the median overall survival of patients with metastatic HER2+ breast cancer has improved. However, management of metastatic disease in the brain and/or CNS – observed in up to 50% of HER2+ breast cancer patients – continues to be a clinical challenge in large part due to the inability of mAbs to sufficiently cross the blood-brain barrier. Although small-molecule inhibitors of HER2 exist and have been clinically approved, their single-agent efficacy in the context of metastatic disease to the brain has been limited. While HER2-targeted therapy in combination with conventional agents has shown some promise for the treatment of patients with metastatic breast cancer, control of brain metastases remains a significant unmet clinical need, as most patients survive less than two years following CNS involvement. Recent advances in cellular immunotherapy approaches have underscored the potential for potent antitumor immune responses and clinical benefit against solid cancers, and these approaches may be effective in the treatment of HER2+ cancers – in particular breast cancer – that have metastasized to the brain. Likewise, HER2 has been suggested as a suitable target for GBM, wherein elevated HER2 protein levels have been correlated with impaired survival.

CAR-based T-cell immunotherapy is being actively investigated for the treatment of solid tumors, including HER2+ cancers. Our academic partners at COH have developed a second-generation HER2-specific CAR T-cell for the treatment of brain and/or leptomeningeal metastases from HER2+ cancers, as well as for the treatment of refractory/relapsed HER2+ GBM. COH's preclinical data demonstrate effective targeting of breast cancer brain metastases with intraventricular delivery of CAR T cells expressing HER2-CARs that contain the 4-1BB costimulatory domain. COH is evaluating the safety of this HER2-specific CAR T cell therapy in two Phase 1 clinical trials that commenced in the fourth quarter of 2018 (ClinicalTrials.gov Identifier: NCT03389230 for HER2+ GBM; ClinicalTrials.gov Identifier: NCT03696030 for HER2+ brain metastases).

MB-105 (PSCA CAR T for Prostate & Pancreatic Cancers)

PSCA is a glycosylphosphatidylinositol-anchored cell membrane glycoprotein. In addition to being highly expressed in the prostate it is also expressed in the bladder, placenta, colon, kidney, and stomach. This gene is upregulated in a large proportion of prostate cancers and is also detected in cancers of the bladder and pancreas. The gene includes a polymorphism that results in an upstream start codon in some individuals; this polymorphism is thought to be associated with a risk for certain gastric and bladder cancers. Prostate cancer may be

amenable to T cell-based immunotherapy since several tumor antigens, including prostate stem-cell antigen ("PSCA"), are widely overexpressed in metastatic disease. Our academic partners at COH have developed a second-generation PSCA-specific CAR T cell therapy that has demonstrated robust *in vitro* and *in vivo* anti-tumor activity in patient-derived, clinically relevant, bone-metastatic prostate cancer xenograft models. COH is evaluating the safety of this PSCA-specific CAR T cell therapy in a Phase 1 trial treating patients with PSCA+ metastatic castration-resistant prostate cancer (ClinicalTrials.gov Identifier: NCT03873805).

In October 2020,we announced initial data from the Phase 1 clinical trial in patients with PSCA-positive castration-resistance prostate cancer ("CRPC"). In a presentation at the 2020 Annual Prostate Cancer Foundation Scientific Retreat, the COH principal investigator reported results from a highly refractory patient treated with MB-105 who experienced a 94 percent reduction in prostate-specific antigen ("PSA"), near complete reduction of measurable soft tissue metastasis by computerized tomography, and improvement in bone metastases by magnetic resonance imaging. We believe additional data could potentially be provided in the second half of 2022.

Technology to Convert GBM from an Immunologically Cold Tumor to an Immunologically Hot Tumor

MB-108 (HSV-1 oncolytic virus C134)

C134 is a next-generation oncolytic herpes simplex virus ("oHSV") that is conditionally replication competent; that is, it can replicate in tumor cells, but not in normal cells, thus killing the tumor cells directly through this process. Replication of C134 in the tumor itself not only kills the infected tumor cells but causes the tumor cell to act as a factory to produce new virus. These virus particles are released as the tumor cell dies and can then proceed to infect other tumor cells in the vicinity and continue the process of tumor kill. In addition to this direct oncolytic activity, the virus promotes an immune response against surviving tumor cells, which increases the antitumor effect of the therapy. The virus expresses a gene from another virus from the same overall virus family, human cytomegalovirus, that allows it to replicate better in the tumor cells than its first-generation predecessors. However, the virus has also been genetically engineered to minimize the production of any toxic effects for the patient receiving the therapy.

To improve this virus over its first-generation predecessors, modifications have focused on improving viral replication and spread within the tumor bed and on enhancing bystander damage to uninfected tumor cells. These effects cumulatively should result in converting an immunologically cold tumor to an immunologically hot tumor, which Mustang anticipates will increase the efficacy of its IL13Rα2-directed CAR T for the treatment of GBM.

UAB is the clinical trial site for the Phase 1 trial of MB-108, and the site has initiated a Phase 1 trial that began enrolling patients in 2019 (ClinicalTrials.gov Identifier: NCT03657576). The primary objective of this study is to determine the safety and tolerability of stereotactic intracerebral injections of escalating doses of MB-108 and to determine the maximally tolerated dose ("MTD") of the oncolytic virus. Secondary objectives are to obtain preliminary information about the potential benefit of MB-108 in the treatment of patients with recurrent malignant gliomas, including relevant data on markers of efficacy, including time to tumor progression and patient survival. In 2021, we intend to combine MB-108 with MB-101 to potentially enhance efficacy in treating GBM. This trial has been on clinical hold since October 2020 due to toxicity at the highest dose level, and UAB expects FDA clearance in the first half of 2021 in order to resume enrolling patients at a lower dose level. As a result of this clinical hold, as well as COVID-19 virus-related accrual delays in 2020, we expect that IND filing for the combination trial of MB-108 with MB-101 (MB-109) will be delayed until the fourth quarter of 2022.

INTELLECTUAL PROPERTY AND PATENTS

General

Our goal is to obtain, maintain and enforce patent protection for our products, formulations, processes, methods and other proprietary technologies, preserve our trade secrets, and operate without infringing on the proprietary rights of other parties, both in the U.S. and in other countries. Our policy is to actively seek to obtain, where appropriate, the broad intellectual property protection for our product candidates, proprietary information and proprietary technology through a combination of contractual arrangements and patents, both in the U.S. and elsewhere in the world.

We also depend upon the skills, knowledge and experience of our scientific and technical personnel, as well as that of our advisors, consultants and other contractors ("know-how"). To help protect our proprietary know-how which is not patentable, and for inventions for which patents may be difficult to enforce, we rely on trade secret protection and confidentiality agreements to protect our interests. To this end, we require all employees, consultants, advisors and other contractors to enter into confidentiality agreements which prohibit the disclosure of confidential information and, where applicable, require disclosure and assignment to us of the ideas, developments, discoveries and inventions that they generate or make, and which are important to our business.

Patents and other proprietary rights are crucial to the development of our business. We will be able to protect our proprietary technologies from unauthorized use by third parties only to the extent that our proprietary rights are covered by valid and enforceable patents, supported by regulatory exclusivity or are effectively maintained as trade secrets. We have a few patents and patent applications related to our compounds and other technology, but we cannot guarantee the scope of protection of the issued patents, or that such patents will survive a validity or enforceability challenge, or that any of the pending patent applications will issue as patents.

Generally, patent applications in the U.S. are maintained in secrecy for a period of 18 months or more. The patent positions of biotechnology and pharmaceutical companies are highly uncertain and involve complex legal and factual questions. Therefore, we cannot predict the breadth of claims allowed in biotechnology and pharmaceutical patents, or their enforceability. To date, there has been no consistent policy regarding the breadth of claims allowed in biotechnology patents. Third parties or competitors may challenge or circumvent our patents or patent applications, if issued. If our competitors prepare and file patent applications in the U.S. that claim technology also claimed by us, we may have to participate in interference or derivation proceedings declared by the U.S. Patent and Trademark Office ("USPTO") to determine priority of invention, which could result in substantial cost, even if the eventual outcome is favorable to us. Because of the extensive time required for development, testing and regulatory review of a potential product, it is possible that before we commercialize any of our products, any related patent may expire or remain in existence for only a short period following commercialization, thus reducing any advantage of the patent. However, the life of a patent covering a product that has been subject to regulatory approval may have the ability to be extended through the patent restoration program, although any such extension could still be minimal. Additionally, statutory caps impose further limitation on any such extensions.

If a patent is issued to a third party containing one or more preclusive or conflicting claims, and those claims are ultimately determined to be valid and enforceable, we may be required to obtain a license, if available, under such patent or to develop or obtain alternative technology. In the event of litigation involving a third party claim, an adverse outcome in the litigation could subject us to significant liabilities to such third party, require us to seek a license for the disputed rights from such third party, and/or require us to cease use of the technology. Further, our breach of an existing license or failure to obtain a license to technology required to commercialize our products may seriously harm our business. We also may need to commence litigation to enforce any patents issued to us or to determine the scope and validity of third party proprietary rights. Litigation would not only involve substantial costs but would also involve substantial time commitments on the part of our key executives and research and development personnel.

In March 2015, we licensed intellectual property related to CAR T technology from COH. The portfolio of rights licensed from COH now includes patents and application directed to CARs targeting IL13Rα2, CD123, CS1, HER2, and PSCA, as well as rights related to modified CAR hinge regions and methods of preparing CAR T cells in particular subpopulations of cell and administering CAR T cells. The intellectual property licensed thereunder relating to IL13Rα2-targeting CARs includes granted patents in the U.S., Australia, China, Europe, Russia, Japan, Hong Kong, Israel, and Mexico, and this patent family further includes pending applications in the U.S., Australia, Brazil, Canada, China, Europe, South Korea, Russia, Japan, Israel, Mexico, and New Zealand. Any patents issuing from the IL13Rα2-targeting CAR will expire no sooner than 2035. The licensed intellectual property relating to relating to CD123-targeting CARs includes issues patents in the U.S., China, Europe, Hong Kong, Israel, Japan, South Korea, and Mexico, and this patent family further includes pending applications in the U.S., Australia, Brazil, China, Europe, Hong Kong, Israel, Japan, South Korea, Mexico, and New Zealand. Any patents issuing from the CD123-targeting CAR will expire no sooner than 2033. The licensed intellectual property relating to relating to CS1-targeting CARs includes issues patents in the U.S., Australia, Israel, and Russia, as well as pending applications in the U.S., Australia, Brazil, Canada, China, Europe, Hong Kong, Israel, South Korea, Mexico, Japan, Russia, and New Zealand. Any patents issuing from the CS1-targeting CAR will expire no sooner than 2035, and some patents relating to particular methods involving CS1-targeting CARs will expire no sooner than 2038. The licensed intellectual property relating to relating to HER2-targeting CARs includes issues patents in Japan and Russia, as well as pending applications in the U.S., Australia, Brazil, Canada, China, Europe, Hong Kong, Israel, Japan, South Korea, Russia, Mexico, and New Zealand. Any patents issuing from the HER2-targeting CAR will expire no sooner than 2036. The licensed intellectual property relating to relating to PSCA-targeting CARs includes issues patents in Europe and Hong Kong, as well as pending applications in the U.S., Australia, Brazil, Canada, China, Europe, Hong Kong, Israel, Japan, South Korea, Russia, and New Zealand. The licensed intellectual property relating to relating modified CAR hinge regions includes issues patents in China, Europe, and Japan, as well as pending applications in the U.S., Australia, China, and Europe. The patents issuing from the modified CAR hinge region family will expire no sooner than 2034. The licensed intellectual property relating to relating to method of preparing or administering CAR T cells includes issues patents in China, Europe, and Japan, as well as pending applications in the U.S., Australia, Brazil, Canada, China, Europe, Hong Kong, Japan, Israel, Mexico, Russia, and New Zealand. The patents relating to these technologies will expire no sooner than 2035 or, in the case of the administration methods, 2036.

Also, in March 2015, we executed a sponsored research agreement with COH, pursuant to which research is performed in the laboratory of Drs. Stephen Forman and Christine Brown. The sponsored research agreement gives us the right to first negotiation under specified maximum terms regarding any future inventions arising from the laboratory.

In May 2017, we licensed intellectual property related to CAR T technology for targeting CD20 from Fred Hutch. The intellectual property includes an international application under the Patent Cooperation Treaty (i.e., a PCT application), which has now matured into several issued patents, including issued patents in the U.S. and Europe, as well as pending applications in the U.S., Australia, Brazil, Canada, China, Europe, Hong Kong, Israel, Japan, South Korea, Mexico, New Zealand, and Russia. These applications contain claims relating to various CD20-targeting CAR constructs and CAR T cells, as well as methods of making and using the same. The national stage applications claiming priority to the PCT application were filed in May 2018 in order to begin substantive examination of the claims. Patents maturing from these national stage applications will expire no sooner than March 2037.

In March 2017, we licensed intellectual property related to antibodies and binding agents that specifically bind to PSCA from the University of California Los Angeles ("UCLA"). The intellectual property includes multiple granted patents and pending applications from around the world including the U.S., Europe, Japan, China, and Canada. The granted patents and patents maturing from the pending applications will expire no sooner than March 2027.

In August 2018, we licensed from St. Jude Children's Research Hospital XSCID Technology related to an *ex vivo* lentiviral vector gene therapy program to provide a normal copy of the *IL2RG* gene to patients born with XSCID.

In February 2019, we licensed Material and Technical Information related to the HSV-1 oncolytic virus C134 from Nationwide in Columbus, Ohio.

In August 2019, we licensed from CSL Behring (Calimmune) the Cytegrity™ stable producer cell line developed and used by St. Jude. The Cytegrity™ stable producer cell line will be used to produce the viral vector for MB-107.

In September 2020, we entered into an exclusive, worldwide licensing agreement with SIRION Biotech for the rights to SIRION's LentiBOOST™ technology for the development of MB-207. This license includes right to granted patents and pending applications in the U.S., Europe, Japan, and Israel. In December 2021 this licensing agreement was amended to include CD20-directed CAR Ts in addition to lentiviral stem cell gene therapy for the treatment of XSCID.

In November 2021, we entered into an exclusive, worldwide licensing agreement with Leiden University Medical Centre for a first-in-class *ex vivo* lentiviral gene therapy for the treatment of RAG1 severe combined immunodeficiency ("RAG1-SCID").

In August 2021, we entered into an exclusive license agreement with Mayo Clinic for a novel technology that may be able to transform the administration of CAR T therapies and potentially allow such therapies to be used as an off-the-shelf therapy.

In addition to the technology the Company has in-licensed, Mustang has also developed its own proprietary intellectual property, both alone and in conjunction with COH. In particular, Mustang filed a U.S. provisional application directed to optimized methods for manufacturing cell-based therapeutics, and Mustang and COH, as co-applicants, filed a U.S. provisional application directed to methods of treating hematological cancers.

In addition to the technology the company has in-licensed, Mustang has also developed its own proprietary intellectual property, both alone and in conjunction with COH. In particular, Mustang owns pending applications in the U.S. and Europe directed to methods for manufacturing cell-based therapeutics, and pending applications in the U.S., Taiwan, and PCT relating to anti-idiotype antibodies. Mustang and COH also own as co-applicants pending application in the U.S., Taiwan, and PCT directed to methods of treating hematological cancers with a combination therapy.

Other Intellectual Property Rights

We depend upon trademarks, trade secrets, knowhow and continuing technological advances to develop and maintain our competitive position. To maintain the confidentiality of trade secrets and proprietary information, we require our employees, scientific advisors, consultants and collaborators, upon commencement of a relationship with us, to execute confidentiality agreements and, in the case of parties other than our research and development collaborators, to agree to assign their inventions to us. These agreements are designed to protect our proprietary information and to grant us ownership of technologies that are developed in connection with their relationship with us. These agreements may not, however, provide protection for our trade secrets in the event of unauthorized disclosure of such information.

In addition to patent protection, we may utilize orphan drug regulations or other provisions of the Food, Drug and Cosmetic Act of 1938, as amended (the "FDCA"), to provide market exclusivity for certain of our product candidates. Orphan drug regulations provide incentives to pharmaceutical and biotechnology companies to develop and manufacture drugs for the treatment of rare diseases, currently defined as

diseases that exist in fewer than 200,000 individuals in the U.S., or diseases that affect more than 200,000 individuals in the U.S. but for which the sponsor does not realistically anticipate will generate a net profit. Under these provisions, a manufacturer of a designated orphan drug can seek tax benefits, and the holder of the first approval of a designated orphan product from the FDA will be granted a seven-year period of marketing exclusivity for such FDA approved orphan product.

LICENSE, CLINICAL TRIAL AND SPONSORED RESEARCH AGREEMENTS

St. Jude Children's Research Hospital

XSCID License

On August 2, 2018, the Company entered into an exclusive worldwide license agreement with St. Jude for the development of a first-in-class *ex vivo* lentiviral gene therapy for the treatment of XSCID. The Company paid $1.0 million in consideration for the exclusive license in addition to an annual maintenance fee of $0.1 million (beginning in 2019). St. Jude is eligible to receive payments totaling $13.5 million upon the achievement of five development and commercialization milestones. Royalty payments in the mid-single digits are due on net sales of licensed products.

XSCID Non-interventional Services Agreement

In December 2019, the Company entered into a Non-Interventional Services Agreement with Children's CGMP, LLC ("Children's"), an affiliate of St. Jude Children's Research Hospital, pursuant to which Children's provides lentiviral vector for non-clinical XSCID research purposes, as well as related advisory services. Pursuant to the agreement, we agreed to fund approximately $0.8 million upon execution of the agreement.

XSCID Data Transfer Agreement

In June 2020, the Company entered into a Data Transfer Agreement for the XSCID program (the "XSCID DTA"). Pursuant to the terms of the XSCID DTA, we made an upfront payment of approximately $1.1 million and will reimburse St. Jude for additional costs in connection with the on-going investigator-initiated study.

City of Hope

In February 2017, the Company and COH amended and restated their license agreement, dated March 17, 2015 (the "Original Agreement"), by entering into three separate amended and restated exclusive license agreements, one relating to the CD123-directed CAR T program, one relating to the IL13Rα2-directed CAR T program, and one relating to the Spacer technology (described below). The total potential consideration payable to COH by the Company, in equity or cash, did not in the aggregate change materially from the Original Agreement. As of December 31, 2021, COH owns 845,385 shares of Class A common stock representing approximately 0.9% of ownership, and has the right to appoint a director to the Board of Directors (the "Board"). The Company considers COH to be a related party, due to the foregoing rights and ownership, as well as the high proportion of the Company's assets that are licensed from COH.

In addition, the Company entered into a sponsored research agreement with COH under which the Company has funded continued research in the amount of $2.0 million per year, payable in four equal installments, which ended in the first quarter of 2020. The research covered under this arrangement is for the IL13Rα2-directed CAR T program, the CD123-directed CAR T program, and the Spacer technology.

CD123 License

In February 2017, the Company entered into an Amended and Restated Exclusive License Agreement with COH to acquire intellectual property rights pertaining to patent rights related to the CD123-directed CAR T program (the "CD123 License"). Pursuant to the CD123 License, the Company and COH acknowledged that an upfront fee had already been paid under the Original Agreement. In addition, COH is eligible to receive an annual maintenance fee of $25,000 and milestone payments totaling up to approximately $14.5 million, upon and subject to the achievement of certain milestones. Royalty payments in the mid-single digits are due on net sales of licensed products. The Company is obligated to pay COH a percentage of certain revenues received in connection with a sublicense ranging from the mid-teens to mid-thirties, depending on the timing of the sublicense in the development of any product. In addition, equity grants made under the Original Agreement were acknowledged, and the anti-dilution provisions of the Original Agreement were carried forward.

CD123 CRA (AML and BPDCN)

In February 2017, the Company entered into a Clinical Research Support Agreement for CD123-directed CAR T program (the "CD123 CRA"). Pursuant to the terms of the CD123 CRA, the Company made an upfront payment of approximately $19,000 and will contribute an additional $97,000 per patient in connection with the on-going investigator-initiated study. Further, the Company agreed to fund approximately $76,000 annually pertaining to the clinical development of the CD123-directed CAR T therapy.

IL13Rα2 License

In February 2017, the Company entered into an Amended and Restated Exclusive License Agreement with COH to acquire intellectual property rights pertaining to patent rights related to the IL13Rα2-directed CAR T program (the "IL13Rα2 License"). Pursuant to the IL13Rα2 License, the Company and COH acknowledged that an upfront fee had already been paid under the Original Agreement. In addition, COH is eligible to receive an annual maintenance fee of $25,000 and milestone payments totaling up to approximately $14.5 million, upon and subject to the achievement of certain milestones. Royalty payments in the mid-single digits are due on net sales of licensed products. The Company is obligated to pay COH a percentage of certain revenues received in connection with a sublicense ranging from the mid-teens to mid-thirties, depending on the timing of the sublicense in the development of any product. In addition, equity grants made under the Original Agreement were acknowledged, and the anti-dilution provisions of the Original Agreement were carried forward.

IL13Rα2 CRA (Glioblastoma)

In February 2017, the Company entered into a Clinical Research Support Agreement for the IL13Rα2-directed CAR T program (the "IL13Rα2 GBM CRA"). Pursuant to the terms of the IL13Rα2 CRA, the Company made an upfront payment of approximately $9,000 and will contribute an additional $140,000 per patient in connection with the on-going investigator-initiated study. Further, the Company agreed to fund approximately $66,000 annually pertaining to the clinical development of the IL13Rα2-directed CAR T therapy.

IL13Rα2 CRA (Leptomeningeal Glioblastoma)

In October 2020, the Company entered into a Clinical Research Support Agreement for the IL13Rα2-directed CAR T program for adult patients with leptomeningeal glioblastoma, ependymoma or medulloblastoma (the "IL13Rα2 Leptomeningeal CRA"). Pursuant to the terms of the IL13Rα2 Leptomeningeal CRA, the Company made an upfront payment of approximately $29,000 and will contribute an additional $150,000 per patient in connection with the on-going investigator-initiated study. Further, the Company agreed to fund approximately $200,000 annually pertaining to the clinical development of the IL13Rα2-directed CAR T therapy.

Sponsored Research Agreement - IL13Rα2 and C134 Combination

In October 2020, the Company entered into a Sponsored Research Agreement ("SRA") with COH to conduct combination studies of a potential IL13Rα2 CAR and C134 oncolytic virus therapy. Pursuant to the SRA, the Company funded research in the amount of $0.3 million for the program.

Spacer License

In February 2017, the Company entered into an Amended and Restated Exclusive License Agreement with COH to acquire intellectual property rights pertaining to patent rights related to Spacer (the "Spacer License"). Pursuant to the Spacer License, the Company and COH acknowledged that an upfront fee had already been paid under the Original Agreement. In addition, COH will receive an annual maintenance fee of $10,000. No royalties are due if the Spacer technology is used in conjunction with a CD123 CAR or an IL13Rα2 CAR, and royalty payments in the low single digits are due on net sales of licensed products if the Spacer technology is used in conjunction with other intellectual property. The Company is obligated to pay COH a percentage of certain revenues received in connection with a sublicense in the mid-thirties, but no such payments are due in connection with sublicenses that are granted in conjunction with the sublicense of other CARs that are licensed from COH to the Company. In addition, equity grants made under the Original Agreement were acknowledged, and the anti-dilution provisions of the Original Agreement were carried forward.

IV/ICV License

In February 2017, the Company entered into an exclusive license agreement (the "IV/ICV License") with COH to acquire intellectual property rights in patent applications related to the intraventricular and intracerebroventricular methods of delivering T cells that express CARs. Pursuant to the IV/ICV License, in March 2017, the Company paid COH an upfront fee of $0.1 million. COH is eligible to receive a

milestone payment totaling approximately $0.1 million, upon and subject to the achievement of a milestone, and an annual maintenance fee. Royalty payments in the low single digits are due on net sales of licensed products. The Company is obligated to pay COH a percentage of certain revenues received in connection with a sublicense in the mid-thirties, but no such payments are due in connection with sublicenses that are granted in conjunction with the sublicense of other CAR T programs that are licensed from COH to the Company.

HER2 Technology License

On May 31, 2017, the Company entered into an exclusive license agreement (the "HER2 Agreement") with COH for the use of HER2 CAR T technology ("HER2 Technology"), which is currently being applied in the treatment of glioblastoma multiforme and in the treatment of HER2+ cancers – in particular breast cancer – that have metastasized to the brain. Pursuant to the HER2 Agreement, the Company paid an upfront fee of $0.6 million and owes an annual maintenance fee of $50,000 (which began in 2019). In addition, COH is eligible to receive milestone payments totaling up to $14.9 million, upon and subject to the achievement of certain milestones. Royalty payments in the mid-single digits are due on net sales of licensed products. The Company is obligated to pay COH a percentage of certain revenues received in connection with a sublicense ranging from the mid-teens to mid-thirties, depending on the timing of the sublicense in the development of any product.

HER2 CRA (HER2+ glioblastoma and HER2+ brain metastases)

In September 2020, the Company entered into a Clinical Research Support Agreement for the HER2-directed CAR T program (the "HER2 CRA"). Pursuant to the terms of the HER2 CRA, the Company made an upfront payment of approximately $29,000 and will contribute an additional $150,000 per patient in connection with the on-going investigator-initiated study. Further, the Company agreed to fund approximately $200,000 annually pertaining to the clinical development of the HER2-directed CAR T therapy.

CS1 Technology License

On May 31, 2017, the Company entered into an exclusive license agreement (the "CS1 Agreement") with COH for the use of CS1-specific CAR T technology ("CS1 Technology"), which is currently being applied in the treatment of multiple myeloma. Pursuant to the CS1 Agreement, the Company paid an upfront fee of $0.6 million and owes an annual maintenance fee of $50,000 (which began in 2019). In addition, COH is eligible to receive milestone payments totaling up to $14.9 million, upon and subject to the achievement of certain milestones. Royalty payments in the mid-single digits are due on net sales of licensed products. The Company is obligated to pay COH a percentage of certain revenues received in connection with a sublicense ranging from the mid-teens to mid-thirties, depending on the timing of the sublicense in the development of any product.

CS1 CRA (multiple myeloma)

In June 2020, the Company entered into a Clinical Research Support Agreement for the CS1-directed CAR T program (the "CS1 CRA"). Pursuant to the terms of the CS1 CRA, the Company made an upfront payment of approximately $32,000 and will contribute an additional $130,000 per patient in connection with the on-going investigator-initiated study. Further, the Company agreed to fund approximately $200,000 annually pertaining to the clinical development of the CS1-directed CAR T therapy.

PSCA Technology License

On May 31, 2017, the Company entered into an exclusive license agreement (the "PSCA Agreement") with COH for the use of PSCA CAR T technology ("PSCA Technology"), which is currently being applied in the treatment of PSCA+ metastatic castration-resistant prostate cancer. Pursuant to the PSCA Agreement, the Company paid an upfront fee of $0.3 million and owes an annual maintenance fee of $50,000 (which began in 2019). In addition, COH is eligible to receive milestone payments totaling up to $14.9 million, upon and subject to the achievement of certain milestones. Royalty payments in the mid-single digits are due on net sales of licensed products. The Company is obligated to pay COH a percentage of certain revenues received in connection with a sublicense ranging from the mid-teens to mid-thirties, depending on the timing of the sublicense in the development of any product.

PSCA CRA

In October 2020, the Company entered into a Clinical Research Support Agreement for the PSCA-directed CAR T program (the "PSCA CRA"). Pursuant to the terms of the PSCA CRA, the Company made an upfront payment of $33,000 and will contribute an additional $125,000 per patient in connection with the on-going investigator-initiated study. Further, the Company agreed to fund approximately $200,000 annually pertaining to the clinical development of the PSCA-directed CAR T therapy.

Manufacturing License

On January 3, 2018, the Company entered into a non-exclusive license agreement with COH to acquire patent and licensed know-how rights related to developing, manufacturing, and commercializing licensed products. The Company paid $75,000 in consideration for the licenses to the patent rights and the licensed know-how in addition to an annual maintenance fee. Royalty payments in the low-single digits are due on net sales of licensed products.

Sponsored Research Agreement - Manufacturing

On January 3, 2018, the Company entered into an SRA with COH to optimize and develop CAR T cell processing procedures. Pursuant to the SRA, the Company funded continued research in the amount of $0.9 million for the program, with an initial term of two (2) years. The SRA expired in January 2020.

University of California License

On March 17, 2017, the Company entered into an exclusive license agreement with the Regents of UCLA (the "UCLA License") to acquire intellectual property rights in patent applications related to the engineered anti-prostate stem cell antigen antibodies for cancer targeting and detection. Pursuant to the UCLA License, the Company paid UCLA the upfront fee of $0.2 million and owes an annual maintenance fee of $15,000 for the first two years, $25,000 for years three and four, and $50,000 per year thereafter. In addition, UCLA is eligible to receive milestone payments totaling up to $14.3 million, upon and subject to the achievement of certain milestones. Royalty payments in the mid-single digits are due on net sales of licensed products.

Fred Hutchinson Cancer Research Center

CD20 Technology License

Effective July 3, 2017, Mustang entered into an exclusive, worldwide licensing agreement with Fred Hutch for the use of a CAR T therapy related to autologous T cells engineered to express a CD20-specific chimeric antigen receptor (the "CD20 Technology License"). Pursuant to the CD20 Technology License, the Company paid Fred Hutch an upfront fee of $0.3 million and owes an annual maintenance fee of $50,000 on each anniversary of the license until the achievement by the Company of regulatory approval of a licensed product using the CD20 Technology. Additional payments are due for the achievement of eleven development milestones totaling $39.1 million. Royalty payments in the mid-single digits are due on net sales of licensed products.

CD20 CTA (NHL and CLL)

Also, on July 3, 2017, in conjunction with the CD20 Technology License from Fred Hutch, Mustang entered into an investigator-initiated clinical trial agreement (the "CD20 CTA") to provide partial funding for a Phase 1/2 clinical trial at Fred Hutch evaluating the safety and efficacy of the CD20 Technology in patients with relapsed or refractory B-cell non-Hodgkin lymphomas ("NHLs"). In connection with the CD20 CTA, the Company agreed to fund up to $5.3 million of costs associated with the clinical trial, which commenced during the fourth quarter of 2017.

In November 2020, the CD20 CTA was amended to include additional funding of approximately $1.8 million for the treatment of five patients with chronic lymphocytic leukemia ("CLL") and other research costs.

Sponsored Research Agreement

On March 17, 2018, the Company entered into an SRA with Fred Hutch related to developing and optimizing processes and systems associated with CD20 cell processing. Pursuant to the SRA, the Company funded continued research in the amount of $0.6 million during the term of the SRA, which expired in March 2019.

Nationwide Children's Hospital License

On February 20, 2019, the Company entered into an exclusive worldwide license agreement with Nationwide for the development of an oncolytic virus (referred to by Nationwide as C134; now referred to by the Company as MB-108) for the treatment of glioblastoma multiforme. The Company paid $0.2 million in consideration for the exclusive license. Nationwide is eligible to receive additional payments

totaling $77.5 million upon the achievement of ten development and commercialization milestones. Royalty payments in the low-single digits are due on net sales of licensed products.

CSL Behring (Calimmune) License

On August 23, 2019, the Company entered into a non-exclusive license agreement with CSL Behring (Calimmune) for the Cytegrity™ stable producer cell line for the production of lentiviral gene therapy for the XSCID gene therapy program. The Cytegrit™ stable producer cell line will be used to produce the viral vector for Mustang's MB-107 and MB-207 lentiviral gene therapies for the treatment of XSCID. The Company paid $0.2 million in consideration for the license. CSL Behring (Calimmune) is eligible to receive additional payments totaling $1.2 million upon the achievement of three development and commercialization milestones. Royalty payments in the low-single digits are due on net sales of licensed products.

SIRION Biotech License

On October 6, 2020, the Company announced a licensing agreement under which we acquired technology rights from SIRION Biotech GmbH ("SIRION") for LentiBOOST™ technology for the development of MB-207, Mustang's lentiviral gene therapy for the treatment of patients with XSCID, who have been previously treated with a hematopoietic stem cell transplantation ("HSCT") and for whom re-treatment is indicated. LentiBOOST™ is SIRION's proprietary non-cytotoxic transduction enhancer for lentiviral vectors.

Pursuant to the agreement, the Company paid SIRION a one-time upfront fee of $0.1 million. In addition, SIRION is eligible to receive additional payments totaling up to approximately $9.1 million upon the achievement of certain development and commercialization milestones. Royalty payments in the low- to mid-single digits are due on aggregate cumulative worldwide net sales of licensed products.

In December 2021 this licensing agreement was amended to include CD20-directed CAR Ts. SIRION is eligible to receive additional payments totaling up to approximately $9.1 million upon the achievement of certain development and commercialization milestones for the additional product.

Minaris Regenerative Medicine Agreement

On November 23, 2020, we announced an agreement with Minaris Regenerative Medicine GmbH ("Minaris") to enable technology transfer and GMP clinical manufacturing in Europe of our MB-107 lentiviral gene therapy program for the treatment of XSCID. Under the terms of the agreement, Minaris will perform technology transfer of the manufacturing and analytical processes, as well as their adoption to the European regulatory environment, for the GMP-compliant manufacturing of the drug product at its site in Ottobrunn, Germany, with the goal of supplying clinical trials in Europe.

Mayo Clinic

CAR T Technology License

On August 12, 2021, we announced that the Company has executed an exclusive license agreement with Mayo Clinic for a novel technology that may be able to transform the administration of CAR T therapies and potentially allow such therapies to be used as an off-the-shelf therapy.

The technology, developed by Larry R. Pease, Ph.D., principal investigator and former director of the Center for Immunology and Immune Therapies at Mayo Clinic, is a new platform to administer CAR T therapy using a two-step approach. First, a peptide is administered to the patient to drive the proliferation of the patient's resident T cells. This is followed by the administration of a viral CAR construct directly into the lymph nodes of the patient. In turn, the viral construct infects the activated T cells and effectively forms CAR T cells *in vivo* in the patient. Successful implementation may lead to an off-the-shelf product with no need to isolate and expand patient T cells *ex vivo.*

Preclinical proof-of-concept has been established, and the ongoing development of this technology will take place at Mayo Clinic. Mustang plans to file an Investigational New Drug ("IND") application for a multicenter Phase 1 clinical trial once a lead construct has been identified.

Pursuant to this agreement, the Company paid an upfront fee of $0.8 million and will pay an annual maintenance fee of $25,000. Additional payments are due for each of two licensed products for the achievement of eleven development and commercial milestones totaling up to $92.6 million per product, and royalty payments in the mid-single digits as a percentage of revenue are due on net sales of licensed products.

Sponsored Research Agreement

In connection with the Mayo Clinic license agreement, the Company entered into an SRA under which the Company will fund research in the amount of $2.1 million over a period of two years. The research performed pursuant to this agreement will support the technology the Company has licensed from Mayo Clinic.

Leiden University Medical Centre

RAG1-SCID Technology License

On November 10, 2021, we announced an exclusive license agreement with Leiden University Medical Centre ("LUMC") for a first-in-class *ex vivo* lentiviral gene therapy for the treatment of RAG1 severe combined immunodeficiency ("RAG1-SCID").

Pursuant to this agreement, the Company paid an upfront fee of $0.4 million. Additional payments are due for the achievement of five development and commercial milestones totaling up to $31.0 million, and royalty payments in the low to mid-single digits as a percentage of revenue are due on net sales of licensed products.

Sponsored Research Agreement

In connection with the RAG1-SCID license, the Company entered into an SRA with LUMC under which the Company will fund research in the amount of 2.3 million euros over a period of five years. The research performed pursuant to this agreement will support the technology the Company has licensed from LUMC.

COMPETITION

Competition in the pharmaceutical and biotechnology industries is intense. Our competitors include pharmaceutical companies and biotechnology companies, as well as universities and public and private research institutions. In addition, companies that are active in different but related fields represent substantial competition for us. Many of our competitors have significantly greater capital resources, larger research and development staffs and facilities and greater experience in drug development, regulation, manufacturing and marketing than we do. These organizations also compete with us to recruit qualified personnel, attract partners for joint ventures or other collaborations, and license technologies that are competitive with ours. To compete successfully in this industry, we must identify novel and unique drugs or methods of treatment and then complete the development of those drugs as treatments in advance of our competitors.

The drugs that we are attempting to develop will have to compete with existing therapies. In addition, a large number of companies are pursuing the development of pharmaceuticals that target the same conditions that we are targeting. Other companies have products or product candidates in various stages of pre-clinical or clinical development, or with marketing approvals, to treat conditions for which we are also seeking to discover and develop product candidates. Some of these potential competing drugs are further advanced in development than our product candidates and may be commercialized earlier.

The field of CAR T therapy is extremely active. Companies and partnerships currently engaged in clinical trials with CAR T modalities include Bristol Myers Squibb, Novartis Pharmaceuticals/University of Pennsylvania, bluebird bio, Allogene Therapeutics, Cellectis, Gilead Sciences, Bellicum Pharmaceuticals, MD Anderson/Ziopharm Oncology, Atara Biotherapeutics, Celyad, Autolus Therapeutics, Precigen and Precision BioSciences.

The gene therapy field is characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. We are aware of companies currently engaged in developing gene therapies in various indications, including Abeona Therapeutics, Adverum Biotechnologies, Astellas, AVROBIO, Axovant Sciences, Biogen, bluebird bio, BioMarin Pharmaceutical, Homology Medicines, Krystal Biotech, MeiraGTx, Novartis Pharmaceuticals, Orchard Therapeutics, Passage Bio, Prevail Therapeutics, REGENXBIO, Rocket Pharmaceuticals, Roche, Sangamo Therapeutics, Sarepta Therapeutics, Solid Biosciences, Ultragenyx Pharmaceuticals, uniQure and Voyager Therapeutics, as well as several companies addressing other methods for delivering or modifying genes and regulating gene expression.

EMPLOYEES

As of December 31, 2021, we had 102 full and part-time employees. None of our employees are represented by a labor union or covered under a collective bargaining agreement and we consider our employee relations to be good. Employees of Fortress also make valuable financial, legal, scientific and other strategic contributions to Mustang on a regular basis.

SUPPLY AND MANUFACTURING

As an early stage development company, we rely on our research partners to manufacture or have manufactured all lentiviral vectors used in the clinical development programs currently in progress at COH, Fred Hutch, St. Jude, the NIH, and LUMC under the IND applications filed by these institutions. In addition we rely on the NIH to produce oncolytic virus for UAB, the clinical trial site for the Phase 1 trial of Nationwide's C134 oncolytic virus (MB-108). We will continue to rely on our research partners to manufacture lentiviral vectors and oncolytic virus for Mustang-IND trials until such time as material is available from our contract manufacturing organizations.

Pursuant to the March 2015 Licensing Agreement with COH, we have the right to make and have made the cellular products, and we have negotiated Investigator-Initiated Clinical Research Support Agreements with COH and Fred Hutch which specify the cell processing costs and numbers of patients which will be supplied under filed protocols. Our research partners have extensive experience manufacturing clinical materials for development studies, but we are currently dependent on both their capacity limitations and continued operating success to manufacture viral vector and to process cells for all CAR T clinical trials for which these partners hold the INDs, as well as to have manufactured oncolytic virus for the MB-108 investigator-IND clinical trial being conducted at UAB.

We have limited experience in processing cells for clinical or commercial purposes. In 2018 we opened our own cell processing facility in Worcester, Massachusetts, in order to manufacture and supply cellular product candidates for all clinical trials that will be conducted under IND applications to be filed by us. In August 2019, the FDA approved our IND application to initiate a multi-center Phase 1/2 clinical trial of MB-102 (CD123 CAR T) and in January 2021, the FDA approved our IND application to initiate a multi-center Phase 2 clinical trial of MB-107 (XSCID). In May 2021, the FDA approved our IND application to initiate a multi-center Phase 1/2 clinical trial of MB-106 (CD-20). As with any supply program, obtaining raw materials of the correct quality cannot be guaranteed, and we cannot ensure that we will be successful in this endeavor.

We expect to rely on contract manufacturing relationships for lentiviral vectors and for the MB-108 oncolytic virus, as well as for any non-CAR T products that we may in-license or acquire in the future for co-administration with our CAR T products. However, there can be no assurance that we will be able to successfully contract with such manufacturers on terms acceptable to us, or at all.

Contract manufacturers for these current and potential future non-CAR T products would be subject to ongoing periodic and unannounced inspections by the FDA, the U.S. Drug Enforcement Administration ("DEA") and corresponding state agencies to ensure strict compliance with the Current Good Manufacturing Practice regulations ("cGMP") and other state and federal regulations. Our contractors, if any, in Europe would face similar challenges from the numerous EU and member state regulatory agencies and authorized bodies. We do not have control over third-party manufacturers' compliance with these regulations and standards, other than through contractual obligations. If they are deemed out of compliance with cGMPs, product recalls could result, inventory could be destroyed, production could be stopped, and supplies could be delayed or otherwise disrupted.

If we need to change manufacturers for these current and potential future non-CAR T products after commercialization, the FDA and corresponding foreign regulatory agencies must approve these new manufacturers in advance, which will involve testing and additional inspections to ensure compliance with FDA regulations and standards and may require significant lead times and delay. Furthermore, switching manufacturers may be difficult because the number of potential manufacturers is limited. It may be difficult or impossible for us to find a replacement manufacturer quickly or on terms acceptable to us, or at all.

GOVERNMENT AND INDUSTRY REGULATIONS

Numerous governmental authorities, principally the FDA and corresponding state and foreign regulatory agencies, impose substantial regulations upon the clinical development, manufacture and marketing of our product candidates, as well as our ongoing research and development activities. None of our product candidates has been approved for sale in any market. Before marketing in the U.S., any drug that we develop must undergo rigorous pre-clinical testing and clinical trials and an extensive regulatory approval process implemented by the FDA under the Food, Drug and Cosmetic Act ("FDCA"). The FDA regulates, among other things, the pre-clinical and clinical testing, safety, efficacy, approval, manufacturing, record keeping, adverse event reporting, packaging, labeling, storage, advertising, promotion, export, and the sale and distribution of biopharmaceutical products.

The regulatory review and approval process is lengthy, expensive and uncertain. We are required to submit extensive preclinical and clinical data and supporting information to the FDA for each indication or use to establish a product candidate's safety and efficacy before we can secure FDA approval to market or sell a product in the U.S. The approval process takes many years, requires the expenditure of substantial resources and may involve ongoing requirements for post-marketing studies or surveillance. Before commencing clinical trials in humans, we must submit an IND to the FDA containing, among other things, preclinical data, chemistry, manufacturing and control information, and an investigative plan. Our submission of an IND may not result in FDA authorization to commence a clinical trial.

The FDA may permit expedited development, evaluation, and marketing of new therapies intended to treat persons with serious or life-threatening conditions for which there is an unmet medical need under its fast track drug development program. A sponsor can apply for fast track designation initially at the time of submission of an IND, or at any time prior to receiving a marketing approval of the new drug application ("NDA") or biologics license application ("BLA"). To receive fast track designation, an applicant must demonstrate:

- that the therapy is intended to treat a serious or life-threatening condition;
- that the therapy is intended to treat a serious aspect of the condition; and
- that the therapy has the potential to address unmet medical needs, and this potential is being evaluated in the planned drug development program.

The FDA must respond to a request for fast track designation within 60 calendar days of receipt of the request. Over the course of development, a product in a fast track development program must continue to meet the criteria for fast track designation. Sponsors of products in fast track drug development programs must be in regular contact with the reviewing division of the FDA to ensure that the evidence necessary to support marketing approval will be developed and presented in a format conducive to an efficient review. Sponsors of products in fast track drug development programs ordinarily are eligible for priority review of a completed application in six months or less and also may be permitted to submit portions of an NDA or BLA to the FDA for review on a rolling basis before the complete application is submitted.

In accordance with the FDCA, sponsors of drugs for serious or life-threatening diseases that fill an unmet medical need may seek approval under the FDA's accelerated approval regulations. Under this authority, the FDA may grant marketing approval for a new drug product on the basis of adequate and well-controlled clinical trials establishing that the drug product has an effect on a surrogate endpoint that is reasonably likely, based on epidemiologic, therapeutic, pathophysiologic, or other evidence, to predict clinical benefit or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity. Approval will be subject to the requirement that the applicant study the drug further to verify and describe its clinical benefit where there is uncertainty as to the relation of the surrogate endpoint to clinical benefit or uncertainty as to the relation of the observed clinical benefit to ultimate outcome. Post-marketing studies are usually underway at the time an applicant files the NDA or BLA. When required to be conducted, such post-marketing studies must also be adequate and well-controlled. The applicant must carry out any such post-marketing studies with due diligence. Many companies who have been granted the right to utilize an accelerated approval approach have failed to obtain approval. Moreover, negative or inconclusive results from the clinical trials we hope to conduct or adverse medical events could cause us to have to repeat or terminate the clinical trials. Accordingly, we may not be able to complete the clinical trials within an acceptable time frame, if at all, and, therefore, could not submit the NDA or BLA to the FDA or foreign regulatory authorities for marketing approval.

Clinical testing must meet requirements for institutional review board oversight, informed consent and good clinical practices, and must be conducted pursuant to an IND, unless exempted.

For purposes of NDA or BLA approval, clinical trials are typically conducted in the following sequential phases:

- *Phase 1*: The drug is administered to a small group of humans, either healthy volunteers or patients, for the first time to test for safety, dosage tolerance, absorption, metabolism, excretion and clinical pharmacology.
- *Phase 2*: Studies are conducted on a larger number of patients to assess the efficacy of the product, to ascertain dose tolerance and the optimal dose range, and to gather additional data relating to safety and potential adverse events.
- *Phase 3*: Studies establish safety and efficacy in an expanded patient population.
- *Phase 4*: The FDA may request phase 4 post-marketing studies to find out more about the drug's long-term risks, benefits, and optimal use, or to test the drug in different patient populations.

The length of time necessary to complete clinical trials varies significantly and may be difficult to predict. Clinical results are frequently susceptible to varying interpretations that may delay, limit or prevent regulatory approvals. Additional factors that can cause delay or termination of our clinical trials, or that may increase the costs of these trials, include:

- slow patient enrollment due to the nature of the clinical trial plan, the proximity of patients to clinical sites, the eligibility criteria for participation in the study or other factors;
- inadequately trained or insufficient personnel at the study site to assist in overseeing and monitoring clinical trials or delays in approvals from a study site's review board;
- longer treatment time required to demonstrate efficacy or determine the appropriate product dose;
- insufficient supply of the product candidates;
- adverse medical events or side effects in treated patients; and
- ineffectiveness of the product candidates.

In addition, the FDA, equivalent foreign regulatory authority, or a data safety monitoring committee for a clinical trial may place a clinical trial on hold or terminate it if it concludes that subjects are being exposed to an unacceptable health risk, or for futility. Any drug is likely to produce some toxicity or undesirable side effects in animals and in humans when administered at sufficiently high doses and/or for a sufficiently long period of time. Unacceptable toxicity or side effects may occur at any dose level at any time in the course of studies in animals designed to identify unacceptable effects of a product candidate, known as toxicological studies, or clinical trials of product candidates. The appearance of any unacceptable toxicity or side effect could cause us or regulatory authorities to interrupt, limit, delay or abort the development of any of our product candidates and could ultimately prevent approval by the FDA or foreign regulatory authorities for any or all targeted indications.

Sponsors of drugs may apply for a special protocol assessment ("SPA") from the FDA for studies intended to form the primary basis of an efficacy claim. The SPA process is a procedure by which the FDA provides official evaluation and written guidance on the design and size of proposed protocols that are intended to form the basis for an NDA or BLA. However, final marketing approval depends on the results of efficacy, the adverse event profile and an evaluation of the benefit/risk of treatment demonstrated in the pivotal clinical trial. Once approved, the SPA may only be changed through a written agreement between the sponsor and the FDA, or in rare cases if the FDA becomes aware of a substantial scientific issue essential to product safety or efficacy the SPA can be rescinded.

Before receiving FDA approval to market a product, we must demonstrate that the product is safe and effective for its intended use by submitting to the FDA an NDA or BLA containing the preclinical and clinical data that have been accumulated, together with chemistry and manufacturing and controls specifications and information, and proposed labeling, among other things. The FDA may refuse to accept an NDA or BLA for filing if certain content criteria are not met and, even after accepting an NDA or BLA, the FDA may require additional information, including clinical data, before approval for marketing a product.

Although uncommon, the FDA may request a Risk Evaluation and Mitigation Strategy, or REMS, as part of an NDA or BLA approval for products with serious safety concerns to help ensure that the benefits of the product outweigh the risks. The REMS plan contains post-marketing obligations of the sponsor to train prescribing physicians, monitor off-label drug use, and perhaps the conduct of Phase 4 follow-up studies and registries to ensure the continued safe use of the drug.

As part of the approval process, the FDA must inspect and approve each manufacturing facility. Among the conditions of approval is the requirement that a manufacturer's quality control and manufacturing procedures conform to cGMP. Manufacturers must expend significant time, money and effort to ensure continued compliance, and the FDA conducts periodic inspections to certify compliance. It may be difficult for our manufacturers or for us to comply with the applicable cGMP, as interpreted by the FDA, and other FDA regulatory requirements. If we, or our contract manufacturers, fail to comply, then the FDA may not allow us to market products that have been affected by the failure.

If the FDA grants approval, the approval will be limited to those conditions and patient populations for which the product is safe and effective, as demonstrated through clinical studies and as reflected in the approved labeling. Further, a product may be marketed only in those dosage forms and for those indications approved in the NDA or BLA. Certain changes to an approved NDA or BLA, including, with certain exceptions, any significant changes to labeling, may require prior approval of a supplemental application before the drug may be marketed as changed. Any products that we manufacture or distribute pursuant to FDA approvals are subject to continuing monitoring and

regulation by the FDA, including compliance with cGMP and the reporting of adverse experiences with the drugs. The nature of marketing claims that the FDA will permit us to make in the labeling and advertising of our products will generally be limited to those specified in FDA approved labeling, and the advertising of our products will be subject to comprehensive monitoring and regulation by the FDA. Drugs whose review was accelerated may carry additional restrictions on marketing activities, including the requirement that all promotional materials are pre-submitted to the FDA. Claims exceeding those contained in the approved labeling will constitute a violation of the FDCA. Violations of the FDCA or regulatory requirements at any time during the product development process, approval process, or marketing and sale following approval may result in agency enforcement actions, including withdrawal of approval, recall, seizure of products, warning letters, injunctions, fines and/or civil or criminal penalties. Any agency enforcement action could have a material adverse effect on our business.

Failure to comply with applicable federal, state and foreign laws and regulations would likely have a material adverse effect on our business. In addition, federal, state and foreign laws and regulations regarding the manufacture and sale of new drugs are subject to future changes.

Other Healthcare Laws and Compliance Requirements

In the U.S., our activities are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare and Medicaid Services (formerly the Health Care Financing Administration), other divisions of the United States Department of Health and Human Services (e.g., the Office of Inspector General), the United States Department of Justice and individual United States Attorney offices within the Department of Justice, and state and local governments.

Pharmaceutical Coverage, Pricing and Reimbursement

In the U.S. and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payors, including government health administrative authorities, managed care providers, private health insurers and other organizations. Third-party payors are increasingly examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy, and, accordingly, significant uncertainty exists as to the reimbursement status of newly approved therapeutics. Adequate third-party reimbursement may not be available for our products to enable us to realize an appropriate return on our investment in research and product development. We are unable to predict the future course of federal or state health care legislation and regulations, including regulations that will be issued to implement provisions of the health care reform legislation enacted in 2010, known as the Affordable Care Act. The Affordable Care Act and further changes in the law or regulatory framework could have a material adverse effect on our business.

International Regulation

In addition to regulations in the U.S., there are a variety of foreign regulations governing clinical trials and commercial sales and distribution of our product candidates. The approval process varies from country to country, and the time may be longer or shorter than that required for FDA approval.

Item 1A. Risk Factors

Investing in our Common Stock or any other type of equity or debt securities we may offer (together, our "Securities") involves a high degree of risk. The following information sets forth risk factors that could cause our actual results to differ materially from those contained in forward-looking statements we have made in this Form 10-K and those we may make from time to time. You should carefully consider the risks described below, in addition to the other information contained in this Form 10-K, before making an investment decision. Our business, financial condition or results of operations could be harmed by any of these risks. The risks and uncertainties described below are not the only ones we face. Additional risks not presently known to us or other factors not perceived by us to present significant risks to our business at this time also may impair our business operations.

Risks Related to Our Finances and Capital Requirements

We have incurred significant losses since our inception. We expect to incur losses for the foreseeable future and may never achieve or maintain profitability. We do not have any products that are approved for commercial sale and therefore do not expect to generate any revenues from product sales in the foreseeable future, if ever.

We are an emerging growth company with a limited operating history. We have focused primarily on in-licensing and developing our product candidates, with the goal of supporting regulatory approval for these product candidates. We have incurred losses since our inception in March 2015 and have an accumulated deficit of $251.8 million as of December 31, 2021. We expect to continue to incur significant operating losses for the foreseeable future. We also do not anticipate that we will achieve profitability for a period of time after generating material revenues, if ever. If we are unable to generate revenues, we will not become profitable and may be unable to continue operations without continued funding.

Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the timing or amount of increased expenses or when or if, we will be able to achieve profitability. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially if:

- one or more of our product candidates receive regulatory approval and are approved for commercial sale, due to our need to establish the necessary commercial infrastructure to launch and commercialize this product candidate without substantial delays, including hiring sales and marketing personnel and contracting with third parties for manufacturing, testing, warehousing, distribution, cash collection and related commercial activities;
- we are required by the FDA or foreign regulatory authorities to perform studies in addition to those currently expected;
- there are any delays in completing our clinical trials or the development of any of our product candidates;
- we execute other collaborative, licensing or similar arrangements that require us to make payments to collaborators or licensors;
- there are variations in the level of expenses related to our future development programs;
- there are any product liability or intellectual property infringement lawsuits in which we may become involved; and
- there are any regulatory developments affecting product candidates of our competitors.

Our ability to become profitable depends upon our ability to generate revenue. To date, we have not generated any revenue from our development stage products, and we do not know when, or if, we will generate any revenue. Our ability to generate revenue depends on a number of factors, including, but not limited to, our ability to:

- obtain regulatory approval for one or more of our product candidates, or any future product candidate that we may license or acquire;
- manufacture or have manufactured commercial quantities of one or more of our product candidates or any future product candidate, if approved, at acceptable cost levels; and
- develop a commercial organization and the supporting infrastructure required to successfully market and sell one or more of our product candidates or any future product candidate, if approved.

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 depress the value of the Company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or even continue our operations. A decline in the value of the Company could also cause you to lose all or part of your investment.

Our short operating history makes it difficult to evaluate our business and prospects.

We have only been conducting operations since our incorporation in March 2015. Our operations to date have been limited to preclinical operations and the in-licensing of our product candidates. We have not yet demonstrated an ability to successfully complete clinical trials, obtain regulatory approvals, manufacture a clinical scale or commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions about our future

performance may not be as accurate as they could be if we had a history of successfully developing and commercializing pharmaceutical products.

In addition, as a young business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to expand our capabilities to support commercial activities. We may not be successful in adding such capabilities.

We expect our financial condition and operating results to continue to fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any past quarterly period as an indication of future operating performance.

We will require substantial additional funding which may not be available to us on acceptable terms, or at all. If we fail to raise the necessary additional capital, we may be unable to complete the development and commercialization of our product candidates, or continue our development programs.

Our operations have consumed substantial amounts of cash since inception. We expect to significantly increase our spending to advance the preclinical and clinical development of our product candidates and launch and commercialize any product candidates for which we may receive regulatory approval, including building our own commercial organizations to address certain markets. We will require additional capital for the further development and, if approved, commercialization of our product candidates, as well as to fund our other operating expenses and capital expenditures. As of December 31, 2021, we had $98.8 million in cash and restricted cash. We cannot provide any assurance that we will be able to raise funds to complete the development of our product candidates. Additionally, we may have to delay or terminate the development of certain product candidates if we are unable to secure additional funding; any such delay or termination, or the announcement of any such delay or termination, may impact our potential growth and have a material adverse effect on the value of our debt and equity securities.

We cannot be certain that additional funding will be available on acceptable terms, or at all. 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, if approved, commercialization of one or more of our product candidates. We may also seek collaborators for one or more of our current or future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available. Any of these events could significantly harm our business, financial condition and prospects.

Our future funding requirements will depend on many factors, including, but not limited to:

- the timing, design and conduct of, and results from, preclinical studies and clinical trials for our product candidates;
- the potential for delays in our efforts to seek regulatory approval for our product candidates, and any costs associated with such delays;
- the costs of establishing a commercial organization to sell, market and distribute our product candidates;
- the rate of progress and costs of our efforts to prepare for the submission of an NDA or BLA for any product candidates that we may in-license or acquire in the future, and the potential that we may need to conduct additional clinical trials to support applications for regulatory approval;
- the costs of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights associated with our product candidates, including any such costs we may be required to expend if our licensors are unwilling or unable to do so;
- the cost and timing of securing sufficient supplies of our product candidates from our contract manufacturers for clinical trials and in preparation for commercialization;
- the effect of competing technological and market developments;
- the terms and timing of any collaborative, licensing, co-promotion or other arrangements that we may establish;
- if one or more of our product candidates are approved, the potential that we may be required to file a lawsuit to defend our patent rights or regulatory exclusivities from challenges by companies seeking to market generic versions of one or more of our product candidates; and
- the success of the commercialization of one or more of our product candidates, if approved.

Future capital requirements will also depend on the extent to which we acquire or invest in additional complementary businesses, products and technologies, although we currently have no commitments or agreements relating to any of these types of transactions.

In order to carry out our business plan and implement our strategy, we anticipate that we will need to obtain additional financing from time to time and may choose to raise additional funds through strategic collaborations, licensing arrangements, public or private equity or debt financing, bank lines of credit, asset sales, government grants, or other arrangements. We cannot be sure that any additional funding, if needed, will be available on terms favorable to us or at all. Furthermore, any additional equity or equity-related financing may be dilutive to our stockholders, and debt or equity financing, if available, may subject us to restrictive covenants and significant interest costs. If we obtain funding through a strategic collaboration or licensing arrangement, we may be required to relinquish our rights to certain of our product candidates or marketing territories.

Our inability to raise capital when needed would harm our business, financial condition and results of operations, and could cause our stock value to decline or require that we wind down our operations altogether.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish proprietary rights.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, grants and license and development agreements in connection with any collaborations. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a stockholder. 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 or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution 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 grant licenses on terms that may not be favorable to us. 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.

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

As a public company, we incur significant legal, accounting and other expenses under the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, as well as rules subsequently implemented by the SEC, and the rules of the Nasdaq Stock Exchange. These rules impose various requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and appropriate corporate governance practices. Our management and other personnel have devoted and will continue to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations increase our legal and financial compliance costs and make some activities more time-consuming and costly. For example, these rules and regulations make it more difficult and more expensive for us to obtain director and officer liability insurance, and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, it may be more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls for financial reporting and disclosure controls and procedures. As a result, we are required to periodically perform an evaluation of our internal controls over financial reporting to allow management to report on the effectiveness of those controls, as required by Section 404 of the Sarbanes-Oxley Act. Additionally, our independent auditors are required to perform a similar evaluation and report on the effectiveness of our internal controls over financial reporting. These efforts to comply with Section 404 and related regulations have required, and continue to require, the commitment of significant financial and managerial resources. While we anticipate maintaining the integrity of our internal controls over financial reporting and all other aspects of Section 404, we cannot be certain that a material weakness will not be identified when we test the effectiveness of our control systems in the future. If a material weakness is identified, we could be subject to sanctions or investigations by the SEC or other regulatory authorities, which would require additional financial and management resources, costly litigation or a loss of public confidence in our internal controls, which could have an adverse effect on the market price of our stock.

Compliance with the Sarbanes-Oxley Act of 2002 will require substantial financial and management resources and may increase the time and costs of completing an acquisition.

A business that we identify as a potential acquisition target may not be in compliance with the provisions of the Sarbanes-Oxley Act regarding the adequacy of internal controls. The development of the internal controls of any such entity to achieve compliance with the Sarbanes-Oxley Act may increase the time and costs necessary to complete any such acquisition. Furthermore, any failure to implement required new or improved controls, or difficulties encountered in the implementation of adequate controls over our financial processes and reporting in the future, could harm our operating results or cause us to fail to meet our reporting obligations. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our securities.

Our ability to use our pre-change NOLs and other pre-change tax attributes to offset post-change taxable income or taxes may be subject to limitation.

We may, from time to time, carry net operating loss carryforwards ("NOLs") as deferred tax assets on our balance sheet. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change" (generally defined as a greater than 50-percentage- point cumulative change (by value) in the equity ownership of certain stockholders over a rolling three-year period), the corporation's ability to use its pre-change NOLs and other pre-change tax attributes to offset its post-change taxable income or taxes may be limited. We may experience ownership changes in the future as a result of shifts in our stock ownership, some of which changes are outside our control. As a result, our ability to use our pre-change NOLs and other pre-change tax attributes to offset post-change taxable income or taxes may be subject to limitation.

Raising funds through lending arrangements may restrict our operations or produce other adverse results.

Our current loan and security agreement (the "Loan Agreement") with Runway Growth Finance Corp. (NASDAQ: RWAY) ("Runway"), which we entered into in March 2022, contains a variety of affirmative and negative covenants, including required financial reporting, limitations on certain dispositions of assets, limitations on the incurrence of additional debt and other requirements. To secure our performance of our obligations under this Loan Agreement, we granted a security interest in substantially all of our assets, other than certain intellectual property assets and certain other excluded collateral, to Runway. Our failure to comply with the covenants in the Loan Agreement, the occurrence of a material impairment in our prospect of repayment or in the perfection or priority of Runway's lien on our assets, as determined by Runway, or the occurrence of certain other specified events could result in an event of default that, if not cured or waived, could result in the acceleration of all or a substantial portion of our debt, potential foreclosure on our assets and other adverse results. Additionally, we are bound by certain negative covenants setting forth actions that are not permitted to be taken during the term of the Loan Agreement without consent of Runway, including, without limitation, incurring certain additional indebtedness, making certain asset dispositions, entering into certain mergers, acquisitions or other business combination transactions or incurring any non-permitted lien or other encumbrance on our assets. The foregoing prohibitions and constraints on our operations could result in our inability to: (i) acquire promising intellectual property or other assets on desired timelines or terms; (ii) reduce costs by disposing of assets or business segments no longer deemed advantageous to retain; (iii) stimulate further corporate growth or development through the assumption of additional debt; or (iv) enter into other arrangements that necessitate the imposition of a lien on corporate assets.

Risks Related to Our Business Strategy, Structure, and Organization

We currently have no products for sale. We are heavily dependent on the success of our product candidates, and we cannot give any assurances that any of our product candidates will receive regulatory approval or be successfully commercialized.

To date, we have invested a significant portion of our efforts and financial resources in the acquisition and development of our product candidates. We have not demonstrated our ability to perform the functions necessary for the successful acquisition, development or commercialization of the technologies we are seeking to develop. As an early stage company, we have limited experience and have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the biopharmaceutical area. Our future success is substantially dependent on our ability to successfully develop, obtain regulatory approval for, and then commercialize such product candidates. Most of our product candidates are currently in early stage clinical trials. Our business depends entirely on the successful development and commercialization of our product candidates, which may never occur. We currently have no drug products for sale, currently generate no revenues from sales of any drug products, and may never be able to develop or commercialize a marketable product.

The successful development, and any commercialization, of our technologies and any product candidates that may occur would require us to successfully perform a variety of functions, including:

- developing our technology platform;
- identifying, developing, formulating, manufacturing and commercializing product candidates;
- entering into successful licensing and other arrangements with product development partners;
- participating in regulatory approval processes, including ultimately gaining approval to market a drug product, which may not occur;
- obtaining sufficient quantities of our product candidates from our third-party manufacturers to meet clinical trial needs and, if approved, to meet commercial demand at launch and thereafter;
- establishing and maintaining agreements with wholesalers, distributors and group purchasing organizations on commercially reasonable terms;
- conducting sales and marketing activities including hiring, training, deploying and supporting our sales force and creating market demand for our product candidates through our own marketing and sales activities, and any other arrangements to promote our product candidates that we may establish; and
- maintaining patent protection and regulatory exclusivity for our product candidates.

Our operations have been limited to organizing the Company, acquiring, developing and securing our proprietary technology and identifying and obtaining preclinical data or clinical data for various product candidates. These operations provide a limited basis for you to assess our ability to continue to develop our technology, identify product candidates, develop and commercialize any product candidates we are able to identify and enter into successful collaborative arrangements with other companies, as well as for you to assess the advisability of investing in our securities. Each of these requirements will require substantial time, effort and financial resources.

Each of our product candidates will require additional clinical development, management of clinical and manufacturing activities, regulatory approval in the jurisdictions in which we plan to market the product, obtaining manufacturing supply, building a commercial organization, and significant marketing efforts before we generate any revenues from product sales, which may not occur. We are not permitted to market or promote any of our product candidates in the U.S. or any other jurisdiction before we receive regulatory approval from the FDA or comparable foreign regulatory authority, respectively, and we may never receive such regulatory approval for any of our product candidates.

Our future growth depends in part on our ability to identify and acquire or in-license products, and if we do not successfully identify and acquire or in-license related product candidates or integrate them into our operations, we may have limited growth opportunities.

An important part of our business strategy is to continue to develop a pipeline of product candidates by acquiring or in-licensing products, businesses or technologies that we believe are a strategic fit with our focus on *ex vivo* lentiviral gene therapy for rare genetic diseases and on novel combinations of CAR T cells with immuno-oncology antibodies, other biologics, and small molecule kinase inhibitors. Future in-licenses or acquisitions, however, may entail numerous operational and financial risks, including, but not necessarily limited to:

- exposure to unknown liabilities;
- disruption of our business and diversion of our management's time and attention to develop acquired products or technologies;
- difficulty or inability to secure financing to fund development activities for such acquired or in-licensed technologies in the current economic environment;
- incurrence of substantial debt or dilutive issuances of securities to pay for acquisitions;
- higher than expected acquisition and integration costs;
- increased amortization expenses;

- difficulty and cost in combining the operations and personnel of any acquired businesses with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and
- inability to retain key employees of any acquired businesses.

We have limited resources to identify and execute the acquisition or in-licensing of third-party products, businesses and technologies and integrate them into our current infrastructure. In particular, we may compete with larger pharmaceutical companies and other competitors in our efforts to establish new collaborations and in-licensing opportunities. These competitors may have access to greater financial resources than us and may have greater expertise in identifying and evaluating new opportunities. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts.

Our approach to the development of our product candidates is unproven, and we do not know whether we will be able to develop any products of commercial value.

Our products candidates are emerging technologies and, consequently, it is conceivable that such technologies may ultimately fail to develop into commercially viable therapies to treat human patients with cancer or other diseases. One of the reasons for the lack of commercial viability could be our inability to obtain regulatory approval for such technologies.

CAR T is a new approach to cancer treatment that presents significant challenges.

We have concentrated much of our research and development efforts on CAR T technology, and our future success is highly dependent on the successful development of T cell immunotherapies in general and our CAR T technology and product candidates in particular. Because CAR T is a new approach to cancer immunotherapy and cancer treatment generally, developing and commercializing our product candidates subjects us to a number of challenges, including, but not necessarily limited to:

- obtaining regulatory approval from the FDA and other regulatory authorities that may have very limited experience with the commercial development of genetically modified T cell therapies for cancer;
- developing and deploying consistent and reliable processes for engineering a patient's T cells ex vivo and infusing the engineered T cells back into the patient;
- conditioning patients with chemotherapy in conjunction with delivering each of our products, which may increase the risk of adverse side effects of our products;
- educating medical personnel regarding the potential side effect profile of each of our products;
- developing processes for the safe administration of these products, including long-term follow-up for all patients who receive our product candidates;
- sourcing clinical and, if approved, commercial supplies for the materials used to manufacture and process our product candidates;
- developing a manufacturing process and distribution network with a cost of goods that allows for an attractive return on investment;
- establishing sales and marketing capabilities after obtaining any regulatory approval to gain market acceptance, and obtaining adequate coverage, reimbursement and pricing by third-party payors and government authorities; and
- developing therapies for types of cancers beyond those addressed by our current 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 research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates

for specific indications may not yield any commercially viable products. If we do not accurately and/or effectively 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.

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 that term is used in the JOBS Act, and may remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary, in 2022, of the completion of the initial public offering of our common stock, (b) in which we have total annual gross revenue of at least $1.07 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our outstanding common stock that are held by non-affiliates exceeds $700 million as of the prior June 30, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three year period. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this Annual Report on Form 10-K;

- 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;

- disclosure obligations regarding executive compensation; and

- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

Further, Section 102(b)(1) of the JOBS Act exempts emerging growth companies from being required to comply with new or revised financial accounting standards until private companies (that is, those that have not had a Securities Act registration statement declared effective or do not have a class of securities registered under the Exchange Act) are required to comply with the new or revised financial accounting standards. The JOBS Act provides that a company can elect to opt out of the extended transition period and comply with the requirements that apply to non-emerging growth companies but any such an election to opt out is irrevocable. We have elected to opt out of such extended transition period which means that when a standard is issued or revised, and it has different application dates for public or private companies, we, as an emerging growth company, will adopt the new or revised standard. This may make comparison of our financial statements with another public company which has opted into using the extended transition period difficult or impossible because of the potential differences in accountant standards used.

We are also a smaller reporting company, and we will remain a smaller reporting company until the fiscal year following the determination that our voting and non-voting common shares held by non-affiliates is more than $250 million measured on the last business day of our second fiscal quarter, or our annual revenues are more than $100 million during the most recently completed fiscal year and our voting and non-voting common shares held by non-affiliates is more than $700 million measured on the last business day of our second fiscal quarter. Similar to emerging growth companies, smaller reporting companies are able to provide simplified executive compensation disclosure, are exempt from the auditor attestation requirements of Section 404, and have certain other reduced disclosure obligations, including, among other things, being required to provide only two years of audited financial statements and not being required to provide selected financial data, supplemental financial information or risk factors.

We have elected to take advantage of certain of the reduced reporting obligations available to us. 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 reduced or more volatile.

Risks Inherent in Drug Development and Commercialization

Delays in the commencement or conduct of our clinical trials could result in increased costs and delay our ability to pursue regulatory approval.

The commencement or conduct of clinical trials can be delayed for a variety of reasons, including, but not necessarily limited to, delays in:

- obtaining regulatory approval to commence a clinical trial;
- identifying, recruiting and training suitable clinical investigators;
- reaching and preserving agreements on acceptable terms with prospective clinical research organizations ("CROs") and trial sites, the terms of which can be subject to extensive negotiation, may be subject to modification from time to time and may vary significantly among different CROs and trial sites;
- obtaining sufficient quantities of a product candidate for use in clinical trials;
- obtaining Institutional Review Board ("IRB") or ethics committee approval to conduct a clinical trial at a prospective site;
- developing and validating companion diagnostics on a timely basis, if required;
- adding new clinical sites once a trial has begun;
- change in the principal investigator or other key staff overseeing the clinical trial at a given site;
- identifying, recruiting and enrolling patients to participate in a clinical trial; or
- retaining (or replacing) patients who have initiated a clinical trial but who may withdraw due to adverse events from the therapy, insufficient efficacy, fatigue with the clinical trial process, personal issues, or other reasons.

Any delays in the commencement of our clinical trials will delay our ability to pursue regulatory approval for product candidates. In addition, many of the factors that cause, or lead to, a delay in the commencement of clinical trials may also ultimately lead to the denial of regulatory approval of a product candidate.

Suspensions or delays in the completion of clinical testing could result in increased costs and delay or prevent our ability to complete development of that product or generate product revenues.

Once a clinical trial has begun, patient recruitment and enrollment may be slower than we anticipate due to the nature of the clinical trial plan, the proximity of patients to clinical sites, the eligibility criteria for participation in the study or other factors. Clinical trials may also be delayed as a result of ambiguous or negative interim results or difficulties in obtaining sufficient quantities of product manufactured in accordance with regulatory requirements and on a timely basis. Further, a clinical trial may be modified, suspended or terminated by us, an IRB, an ethics committee or a data safety monitoring committee overseeing the clinical trial, any clinical trial site with respect to that site, or the FDA or other regulatory authorities, due to a number of factors, including, but not necessarily limited to:

- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- inspection of the clinical trial operations or clinical trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold;
- stopping rules contained in the protocol;
- unforeseen safety issues or any determination that the clinical trial presents unacceptable health risks; and
- lack of adequate funding to continue the clinical trial.

Changes in regulatory requirements and guidance also may occur, and we may need to amend clinical trial protocols to reflect these changes. Amendments may require us to resubmit clinical trial protocols to IRBs for re-examination, which may in turn impact the costs and timing

of, and the likelihood of successfully completing, a clinical trial. If we experience delays in the completion of, or if we must suspend or terminate, any clinical trial of any product candidate, our ability to obtain regulatory approval for that product candidate will be delayed, and the commercial prospects, if any, for the product candidate may suffer as a result. In addition, many of these factors may also ultimately lead to the denial of regulatory approval of a product candidate.

Product candidates that we advance into clinical trials may not receive regulatory approval.

Pharmaceutical development has inherent risks. We will be required to demonstrate through well-controlled clinical trials that product candidates are effective with a favorable benefit-risk profile for use in their target indications before seeking regulatory approvals for their commercial sale. Success in early clinical trials does not mean that later clinical trials will be successful, as product candidates in later-stage clinical trials may fail to demonstrate sufficient safety or efficacy despite having progressed through initial clinical testing. Also, we may need to conduct additional clinical trials that are not currently anticipated. Companies frequently suffer significant setbacks in advanced clinical trials, even after earlier clinical trials have shown promising results. As a result, product candidates that we advance into clinical trials may not receive regulatory approval.

In addition, even if our product candidates were to obtain approval, regulatory authorities may approve any such product candidates or any future product candidate for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. The regulatory authority may also require the label to contain warnings, contraindications, or precautions that limit the commercialization of the product. In addition, the DEA (or foreign equivalent) may classify one or more of our product candidates in scheduling under the Controlled Substances Act (or its foreign equivalent) that could impede such product's commercial viability. Any of these scenarios could impact the commercial prospects for one or more of our current or future product candidates.

Any product candidates we advance into clinical development are subject to extensive regulation, which can be costly and time consuming, cause unanticipated delays or prevent the receipt of the required approvals to commercialize product candidates.

The research and clinical development, testing, manufacturing, labeling, storage, record-keeping, advertising, promotion, import, export, marketing and distribution of any product candidate, including our product candidates, is subject to extensive regulation by the FDA in the United States and by comparable health authorities in foreign markets. In the United States, we are not permitted to market a product candidate until such product candidate's Biologics License Application ("BLA") or NDA is approved by the FDA. The process of obtaining approval is uncertain, expensive, often spanning many years, and can vary substantially based upon the type, complexity and novelty of the products involved. In addition to significant and expensive clinical testing requirements, our ability to obtain marketing approval for product candidates depends on obtaining the final results of required non-clinical testing, including characterization of the manufactured components of our product candidates and validation of our manufacturing processes. The FDA may determine that our product manufacturing processes, testing procedures or equipment and facilities are inadequate to support approval. Approval policies or regulations may change, and the FDA has substantial discretion in the pharmaceutical approval process, including the ability to delay, limit or deny approval of a product candidate for many reasons. Despite the time and expense invested in the clinical development of product candidates, regulatory approval is never guaranteed.

The FDA and other regulatory agencies can delay, limit or deny approval of a product candidate for many reasons, including, but not limited to:

- the FDA or comparable foreign regulatory authorities may disagree with the trial design or implementation of our clinical trials, including proper use of clinical trial methods and methods of data analysis;
- an inability to establish sufficient data and information to demonstrate to the satisfaction of the FDA that a product candidate is safe and effective for an indication;
- the FDA may not accept clinical data from trials conducted by individual investigators or in countries where the standard of care is potentially different from that of the United States;
- the results of clinical trials may not meet the level of statistical significance required by the FDA for approval;
- the FDA may disagree with the interpretation of data from preclinical studies or clinical trials;

- the FDA may determine that our manufacturing processes or facilities or those of third-party manufacturers with which we or our respective collaborators currently contract for clinical supplies and plan to contract for commercial supplies do not satisfactorily comply with CGMPs; or

- the approval policies or interpretation of regulations of the FDA may significantly change in a manner rendering the clinical data insufficient for approval or the product characteristics or benefit-risk profile unfavorable for approval.

With respect to foreign markets, approval procedures vary among countries and, in addition to the aforementioned risks, can involve additional product testing, administrative review periods and agreements with pricing authorities. In addition, rapid drug and biological development during the COVID-19 pandemic has raised questions about the safety and efficacy of certain marketed pharmaceuticals and may result in increased cautiousness by the FDA and comparable foreign regulatory authorities in reviewing new pharmaceuticals based on safety, efficacy or other regulatory considerations and may result in significant delays in obtaining regulatory approvals. Any delay in obtaining, or inability to obtain, applicable regulatory approvals would prevent us from commercializing our product candidates.

Regulatory approval for our product candidates by the FDA, or any similar regulatory authorities outside the United States, is limited to those specific indications and conditions for which clinical safety and efficacy have been demonstrated.

Any regulatory approval is limited to the indications for use and related treatment of those specific diseases and indications set forth in the approval for which a product is deemed to be safe and effective by the FDA, or other similar regulatory authorities outside the United States. In addition to the regulatory approval required for new drug products, new formulations or indications for an approved product also require regulatory approval. If we are not able to obtain regulatory approval for any desired future indications for our products, our ability to effectively market and sell our products may be reduced and our business may be adversely affected.

While physicians may choose to prescribe drugs for uses that are not described in the product's labeling and for uses that differ from those tested in clinical studies and approved by the regulatory authorities ("off-label uses"), our ability to promote the products is limited to those indications that are specifically approved by the FDA, or similar regulatory authorities outside the United States. Such off-label uses are common across medical specialties and may constitute an appropriate treatment for some patients in certain circumstances. Regulatory authorities in the U.S. generally do not regulate practice of medicine or the behavior of physicians in their choice of treatments. Regulatory authorities do, however, restrict communications by pharmaceutical companies on the promotion of off-label use. If our promotional activities fail to comply with these regulations or guidelines, we may be subject to compliance or enforcement actions, including Warning Letters, by these authorities. In addition, our failure to follow FDA laws, regulations and guidelines relating to promotion and advertising may cause the FDA to suspend or withdraw an approved product from the market, request a recall or institute fines or penalties, or could result in disgorgement of money, operating restrictions, corrective advertising, injunctions or criminal prosecution, any of which could harm our business.

If any of our product candidates is approved and we or our contract manufacturer(s) fail to produce the product, or components of the product, in the volumes that we require on a timely basis, or fail to comply with stringent regulations applicable to pharmaceutical drug manufacturers, we may face delays in the commercialization of our product candidates or be unable to meet market demand, and may lose potential revenues.

The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls, and the use of specialized processing equipment. We may enter into development and supply agreements with contract manufacturers for the completion of pre-commercialization manufacturing development activities and, if approved, the manufacture of commercial supplies for one or more of our product candidates. Any termination or disruption of our relationships with our contract manufacturers may materially harm our business and financial condition and frustrate any commercialization efforts for each respective product candidate.

All of our contract manufacturers must comply with strictly enforced federal, state and foreign regulations, including current good manufacturing practice ("cGMP") requirements enforced by the FDA through its establishment inspection program. We are required by law to establish adequate oversight and control over raw materials, components and finished products furnished by our third-party suppliers and contract manufacturers, but we have little control over their compliance with these regulations. Any failure to comply with applicable regulations may result in fines and civil penalties, suspension of production, restrictions on imports and exports, suspension or delay in product approval, product seizure or recall, or withdrawal of product approval, and would limit the availability of our product and customer confidence in our product. Any manufacturing defect or error discovered after products have been produced and distributed could result in even more significant consequences, including costly recalls, re-stocking costs, damage to our reputation and potential for product liability claims.

If the contract manufacturers upon whom we may rely to manufacture one or more of our product candidates, and any future product candidate we may in-license, fails to deliver the required commercial quantities on a timely basis at commercially reasonable prices, we would likely be unable to meet demand for our products and we would lose potential revenues.

If serious adverse or unacceptable side effects are identified during the development of one or more of our product candidates or any future product candidate, we may need to abandon or limit our development of some of our product candidates.

If one or more of our product candidates or any future product candidate are associated with undesirable side effects or adverse events in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit development to more narrow uses or subpopulations in which the adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. In our industry, many compounds that initially showed promise in early stage testing have later been found to cause serious adverse events that prevented further development of the compound. In the event that our clinical trials reveal a high or unacceptable severity and prevalence of adverse events, our trials could be suspended or terminated, and the FDA or comparable foreign regulatory authorities could order us to cease further development or deny approval of one or more of our product candidates or any future product candidate for any or all targeted indications. The FDA could also issue a letter requesting additional data or information prior to making a final decision regarding whether or not to approve a product candidate. The number of requests for additional data or information issued by the FDA in recent years has increased and has resulted in substantial delays in the approval of several new drugs. Adverse events or undesirable side effects caused by one or more of our product candidates or any future product candidate could also result in the inclusion of unfavorable information in our product labeling or in denial of regulatory approval by the FDA or other regulatory authorities for any or all targeted indications, which would, in turn, prevent us from commercializing and generating market acceptance and revenues from the sale of that product candidate. Adverse events or side effects could affect patient recruitment or the ability of enrolled patients to complete the trial and could result in potential product liability claims.

Additionally, if one or more of our product candidates or any future product candidate receives marketing approval and we or others later identify undesirable side effects caused by this product, a number of potentially significant negative consequences could result, including:

- regulatory authorities may require the addition of unfavorable labeling statements, including specific warnings , black box warnings, adverse reactions, precautions, and/or contraindications;
- regulatory authorities may suspend or withdraw their approval of the product, and/or require it to be removed from the market;
- we may be required to change the way the product is administered, conduct additional clinical trials or change the labeling of the product; or
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of any of our product candidates or any future product candidate or could substantially increase our commercialization costs and expenses, which in turn could delay or prevent us from generating significant revenues, or any revenues, from its sale.

Even if one or more of our product candidates receives regulatory approval, it and any other products we may market will remain subject to substantial regulatory scrutiny.

If one or more of our product candidates that we may license or acquire is approved, the approved product candidate will be subject to ongoing requirements and review by the FDA and other regulatory authorities. These requirements include labeling, packaging, storage, advertising, promotion, record-keeping and submission of safety and other post-market information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping of the drug, and requirements regarding our presentations to and interactions with health care professionals.

The FDA, or other regulatory authorities, may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. The FDA and other applicable regulatory authorities closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and other applicable regulatory authorities impose stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our products for only their approved indications, we may be subject to enforcement action

for off-label marketing. Violations of the FDCA relating to the promotion of prescription drugs may lead to investigations, civil claims, and/or criminal charges alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products, operations, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters, untitled letters, import alerts, and/or inspection observations;
- 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;
- suspension or withdrawal of marketing or regulatory approvals;
- suspension of any ongoing clinical trials;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions, consent decrees, and/or the imposition of civil or criminal penalties.

The FDA's policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates, or negatively affect those products for which we may have already received regulatory approval, if any. 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 the various actions listed above, including losing any marketing approval that we may have obtained.

We will need to obtain FDA approval of any proposed product brand names, and any failure or delay associated with such approval may adversely impact our business.

A pharmaceutical product cannot be marketed in the U.S. or other countries until we have completed a rigorous and extensive regulatory review process, including approval of a brand name. Any brand names we intend to use for our product candidates will require approval from the FDA regardless of whether we have secured a formal trademark registration from the USPTO. The FDA typically conducts a review of proposed product brand names, including an evaluation of the potential for confusion with other product names. The FDA may also object to a product brand name if it believes the name inappropriately implies medical claims. If the FDA objects to any of our proposed product brand names, we may be required to adopt an alternative brand name for our product candidates. If we adopt an alternative brand name, we would lose the benefit of our existing trademark applications for such product candidate and may be required to expend significant additional resources in an effort to identify a suitable product brand name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. We may be unable to build a successful brand identity for a new trademark in a timely manner or at all, which would limit our ability to commercialize our product candidates.

Public concern regarding the safety of drug products could delay or limit our ability to obtain regulatory approval, result in the inclusion of unfavorable information in our labeling, or require us to undertake other activities that may entail additional costs.

In light of widely publicized events concerning the safety risk of certain drug products, the FDA, members of the U.S. Congress, the Government Accountability Office, medical professionals and the general public have raised concerns about potential drug safety issues. These events have resulted in the withdrawal of drug products, revisions to drug labeling that further limit use of the drug products and the establishment of risk management programs. The Food and Drug Administration Amendments Act of 2007, or FDAAA, grants significant expanded authority to the FDA, much of which is aimed at improving the safety of drug products before and after approval. In particular, the new law authorizes the FDA to, among other things, require post-approval studies and clinical trials, mandate changes to drug labeling to reflect new safety information and require risk evaluation and mitigation strategies for certain drugs, including certain currently approved drugs. It also significantly expands the federal government's clinical trial registry and results databank, which we expect will result in significantly increased government oversight of clinical trials. Under the FDAAA, companies that violate these and other provisions of the new law are subject to substantial civil monetary penalties, among other regulatory, civil and criminal penalties. The increased attention to drug safety issues may result in a more cautious approach by the FDA in its review of data from our clinical trials. Data from clinical trials may receive greater scrutiny, particularly with respect to safety, which may make the FDA or other regulatory authorities more likely to require additional preclinical studies or clinical trials. If the FDA requires us to conduct additional preclinical studies or clinical trials prior to approving any of our product candidates, our ability to obtain approval of this product candidate will be delayed. If the FDA requires us to provide additional clinical or preclinical data following the approval of any of our product candidates, the indications for which this product candidate is approved may be limited or there may be specific warnings or limitations on dosing, and our efforts to commercialize our product candidates may be otherwise adversely impacted.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for one or more of 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 or similar regulatory authorities outside the United States. Some of our competitors have ongoing clinical trials for product candidates that treat the same indications that we are targeting for our product candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates. Available therapies for the indications we are pursuing can also affect enrollment in our clinical trials. Patient enrollment is affected by other factors including, but not necessarily limited to:

- the severity of the disease under investigation;
- the eligibility criteria for the study in question;
- the perceived risks and benefits of the product candidate under study;
- the efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the number of clinical trials sponsored by other companies for the same patient population;
- the ability to monitor patients adequately during and after treatment; and
- the proximity and availability of clinical trial sites for prospective patients.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our product candidates or future product candidates, which would cause the value of the Company to decline and limit our ability to obtain additional financing.

If our competitors develop treatments for any of our product candidates' target indications and those competitor products are approved more quickly, marketed more successfully or demonstrated to be more effective, the commercial opportunity for our product candidate will be reduced or eliminated.

The biotechnology and pharmaceutical industries are subject to rapid and intense technological change. We face, and will continue to face, competition in the development and, if approved, marketing of our product candidates from academic institutions, government agencies, research institutions and biotechnology and pharmaceutical companies. There can be no assurance that developments by others will not render one or more of our product candidates obsolete or noncompetitive. Furthermore, new developments, including the development of other drug technologies and methods of preventing the incidence of disease, occur in the pharmaceutical industry at a rapid pace. These developments may render one or more of our product candidates obsolete or noncompetitive.

Competitors may seek to develop alternative formulations that do not directly infringe on our in-licensed patent rights. The commercial opportunity for one or more of our product candidates could be significantly harmed if competitors are able to develop alternative formulations outside the scope of our in-licensed patents. Compared to us, many of our potential competitors have substantially greater:

- capital resources;
- development resources, including personnel and technology;
- clinical trial experience;
- regulatory experience;
- expertise in prosecution of intellectual property rights; and
- manufacturing, distribution and sales and marketing experience.

As a result of these factors, our competitors may obtain regulatory approval of their products more rapidly than we are able to or may obtain patent protection or other intellectual property rights that limit our ability to develop or commercialize one or more of our product candidates. Our competitors may also develop drugs that are more effective, safe, useful and less costly than ours and may be more successful than us in manufacturing and marketing their products. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. We will also face competition from these third parties in establishing clinical trial sites, in patient registration for clinical trials, and in identifying and in-licensing new product candidates.

Further, generic therapies are typically sold at lower prices than branded therapies and are generally preferred by hospital formularies and managed care providers of health services. We anticipate that, if approved, our product candidates will face increasing competition in the form of generic versions of branded products of competitors, including those that have lost or will lose their patent exclusivity. In the future, we may face additional competition from a generic form of our own candidates when the patents covering them begin to expire, or earlier if the patents are successfully challenged. If we are unable to demonstrate to physicians and payers that the key differentiating features of our product candidates translate to overall clinical benefit or lower cost of care, we may not be able to compete with generic alternatives.

If any of our product candidates are successfully developed but do not achieve broad market acceptance among physicians, patients, healthcare payors and the medical community, the revenues that any such product candidates generate from sales will be limited.

Even if our product candidates receive regulatory approval, they may not gain market acceptance among physicians, patients, healthcare payors and the medical community. Coverage and reimbursement of our product candidates by third-party payors, including government payors, generally would also be necessary for commercial success. The degree of market acceptance of any approved products would depend on a number of factors, including, but not necessarily limited to:

- the efficacy and safety as demonstrated in clinical trials;
- the timing of market introduction of such product candidate as well as competitive products;
- the clinical indications for which the product is approved;
- acceptance by physicians, major operators of cancer clinics and patients of the product as a safe and effective treatment;

- the safety of such product candidates seen in a broader patient group, (i.e., based on actual use);
- the availability, cost and potential advantages of alternative treatments, including less expensive generic drugs;
- the availability of adequate reimbursement and pricing by third-party payors and government authorities;
- changes in regulatory requirements by government authorities for our product candidates;
- the relative convenience and ease of administration of the product candidate for clinical practices;
- the product labeling or product insert required by the FDA or regulatory authority in other countries, including any contradictions, warnings, drug interactions, or other precautions;
- changes in the standard of care for the targeted indications for our product candidate or future product candidates, which could reduce the marketing impact of any labeling or marketing claims that we could make following FDA approval;
- the approval, availability, market acceptance and reimbursement for a companion diagnostic, if any;
- the prevalence and severity of adverse side effects; and
- the effectiveness of our sales and marketing efforts.

If any product candidate that we develop does not provide a treatment regimen that is as beneficial as, or is not perceived as being as beneficial as, the current standard of care or otherwise does not provide patient benefit, that product candidate, if approved for commercial sale by the FDA or other regulatory authorities, likely will not achieve market acceptance. Our ability to effectively promote and sell any approved products will also depend on pricing and cost-effectiveness, including our ability to produce a product at a competitive price and our ability to obtain sufficient third-party coverage or reimbursement. If any product candidate is approved but does not achieve an adequate level of acceptance by physicians, patients and third-party payors, our ability to generate revenues from that product would be substantially reduced. In addition, our efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources, may be constrained by FDA rules and policies on product promotion, and may never be successful.

Reimbursement may be limited or unavailable in certain market segments for our product candidates, which could make it difficult for us to sell our products profitably.

There is significant uncertainty related to the third-party coverage and reimbursement of newly approved drugs. Such third-party payors include government health programs such as Medicare, managed care providers, private health insurers and other organizations. We intend to seek approval to market our product candidates in the U.S., the EU and other selected foreign jurisdictions. Market acceptance and sales of our product candidates in both domestic and international markets will depend significantly on the availability of adequate coverage and reimbursement from third-party payors for any of our product candidates and may be affected by existing and future health care reform measures. Government and other third-party payors are increasingly attempting to contain healthcare costs by limiting both coverage and the level of reimbursement for new drugs and, as a result, they may not cover or provide adequate payment for our product candidates. These payors may conclude that our product candidates are less safe, less effective or less cost-effective than existing or future introduced products, and third-party payors may not approve our product candidates for coverage and reimbursement or may cease providing coverage and reimbursement for these product candidates.

Obtaining coverage and reimbursement approval for a product from a government or other third-party payor is a time consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data for the use of our products. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If reimbursement of our future products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, we may be unable to achieve or sustain profitability.

In some foreign countries, particularly in the EU, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product candidate. To obtain reimbursement or pricing approval in some countries, we may be required to conduct additional clinical trials that compare the cost-effectiveness of our product candidates to other available therapies. If reimbursement of our product candidates is unavailable or limited in scope or amount in a particular country, or if pricing is set at unsatisfactory levels, we may be unable to achieve or sustain profitability of our products in such country.

If we are unable to establish sales, marketing and distribution capabilities or to enter into agreements with third parties to market and sell our product candidates, we may be unsuccessful in commercializing our product candidates, if they are approved.

We currently do not have a marketing or sales organization for the marketing, sales and distribution of pharmaceutical products. In order to commercialize any approved product candidate, we would need to build marketing, sales, distribution, managerial and other non-technical capabilities or arrange for third parties to perform these services, and we may be unsuccessful in doing so. In the event of successful development and regulatory approval of any of our current or future product candidates, we expect to build a targeted specialist sales force to market or co-promote the product. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include, but are not necessarily limited to:

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

We face potential product liability exposure, and if successful claims are brought against us, we may incur substantial liability for one or more of our product candidates or a future product candidate we may license or acquire and may have to limit their commercialization.

The use of one or more of our product candidates and any future product candidate we may license or acquire in clinical trials and the sale of any products for which we obtain marketing approval expose us to the risk of product liability claims. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, 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. Product liability claims might be brought against us by consumers, health care providers or others using, administering or selling our products. If we cannot successfully defend ourselves against these claims, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- withdrawal of clinical trial participants;
- suspension or termination of clinical trial sites or entire trial programs;
- decreased demand for any product candidates or products that we may develop;
- initiation of investigations by regulators;
- impairment of our business reputation;
- costs of related litigation;
- substantial monetary awards to patients or other claimants;
- loss of revenues;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize our product candidate or future product candidates.

We will obtain limited product liability insurance coverage for any and all of our upcoming clinical trials. However, our insurance coverage may not reimburse us or may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and, in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. When needed we intend to expand our insurance coverage to include the sale of commercial products if we obtain marketing approval for one or more of our product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance for any products approved for marketing. On occasion, large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us could cause our stock price to fall and, if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business.

Product candidates, even if successfully developed and commercialized, may be effective only in combating certain specific types of cancer, and the market for drugs designed to combat such cancer type(s) may be small and unprofitable.

There are many different types of cancer, and a treatment that is effective against one type of cancer may not be effective against another. CAR T or other technologies we pursue may only be effective in combating specific types of cancer but not others. Even if one or more of our products proves to be an effective treatment against a given type of cancer, the number of patients suffering from such cancer may be small, in which case potential sales from a therapy designed to combat such cancer would be limited.

Our gene therapy product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval.

We have concentrated a portion of our therapeutic product research and development efforts on our gene therapy platform, and our future success depends, in part, on the successful development of this therapeutic approach. There can be no assurance that any development problems we experience in the future related to our gene therapy platform will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays in developing a sustainable, reproducible and commercial-scale manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical studies or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical study requirements of the FDA, the European Medicines Agency, or EMA, and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Currently, a limited number of gene therapy products, including CAR T therapies, have been approved by the FDA, the EMA and the European Commission. Given the few precedents of approved gene therapy products, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the EU or other jurisdictions. Approvals by the EMA and the European Commission may not be indicative of what the FDA may require for approval.

Regulatory requirements governing the development of gene therapy products have changed frequently and may continue to change in the future. The FDA has established the Office of Tissues and Advanced Therapies within the Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and to advise the CBER on its review. The FDA can put an investigational new drug application, or IND, on clinical hold if the information in an IND is not sufficient to assess the risks in pediatric patients. Before a clinical study can begin at any institution, that institution's IRB and its Institutional Biosafety Committee will have to review the proposed clinical study to assess the safety of the study. 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.

These regulatory review agencies, committees and advisory groups and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, 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 studies, limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

Negative public opinion and increased regulatory scrutiny of the therapies that underpin many of our product candidates may damage public perception of our product candidates or adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Public perception may be influenced by claims that one or more of the therapies underpinning our product candidates, including without limitation gene therapy, is unsafe, and such therapy may not gain the acceptance of the public or the medical community. In particular, the success of our gene therapy platforms will depend upon physicians specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our 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. 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 and commercialization of our product candidates or demand for any products we may develop. Adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity, could lead to increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our potential product candidates, stricter labeling requirements for those product candidates that do obtain approval and/or a decrease in demand for any such product candidates. Concern about environmental spread of our products, whether real or anticipated, may also hinder the commercialization of our products.

Risks Related to Reliance on Third Parties

We rely, and expect to continue to rely, on third parties to conduct our preclinical studies and clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials or complying with applicable regulatory requirements.

We rely on our licensors to conduct some of our preclinical studies and some of our clinical trials for our product candidates and for future product candidates, and we rely on third-party contract research organizations and site management organizations to conduct most of the remainder of our preclinical studies and all the rest of our clinical trials. We expect to continue to rely on third parties, such as our licensors, contract research organizations, site management organizations, clinical data management organizations, medical institutions and clinical investigators, to conduct some of our preclinical studies and all of our clinical trials. The agreements with these third parties might terminate for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, that could delay our product development activities.

Our reliance on these third parties for research and development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our preclinical studies and clinical trials are conducted in accordance with the general investigational plan and protocols for the trial and for ensuring that our preclinical studies are conducted in accordance with good laboratory practices ("GLPs") as appropriate. Moreover, the FDA requires us to comply with standards, commonly referred to as good clinical practices ("GCPs") 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. Regulatory authorities enforce these requirements through periodic inspections of trial sponsors, clinical investigators and trial sites. If we or any of our CROs fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a regulatory authority, such regulatory authority will determine that any of our clinical trials complies with GCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process. 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 specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

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

If any of our relationships with these third-party contract research organizations or site management organizations terminates, we may not be able to enter into arrangements with alternative contract research organizations or site management organizations or to do so on commercially reasonable terms. Switching or adding additional contract research organizations or site management organizations involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new contract research organization or site management organization commences work. As a result, delays could occur, which could compromise our ability to

meet our desired development timelines. Though we carefully manage our relationships with our contract research organizations or site management organizations, there can be no assurance that we will not encounter similar challenges or delays in the future. Forces beyond our control, including the impacts of COVID-19, could disrupt the ability of our third-party CROs, site management organizations, clinical data management organizations, medical institutions, and clinical investigators to conduct our preclinical studies and our clinical trials for our product candidates and for any future product candidate. For instance, situations like the coronavirus disease outbreak have the potential to adversely impact our product development activities. At this time, the impact of the coronavirus disease outbreak is not having a material adverse effect on our business, but no assurance can be given it will not in the future if the situation persists.

We are currently reliant on the City of Hope National Medical Center, the Fred Hutchinson Cancer Research Center, St. Jude Children's Research Hospital, the University of Alabama at Birmingham, Leiden University Medical Centre, and Mayo Clinic for a substantial portion of our research and development efforts and the early clinical testing of our product candidates.

A substantial portion of our research and development has been and will continue to be conducted by COH, Fred Hutch, St. Jude, UAB, LUMC and Mayo Clinic pursuant to a sponsored research agreement and/or clinical trial agreements with each of those parties. As a result, our future success is heavily dependent on the results of research and development efforts of Dr. Stephen Forman and his team at COH, of Drs. Brian Till and Mazyar Shadman and their team at Fred Hutch, of Drs. Stephen Gottschalk and Ewelina Mamcarz and their team at St. Jude, of Dr. James M. Markert and his team at UAB, of Dr. Frank J. Staal and his team at LUMC, and of Dr. Larry R. Pease and his team at Mayo Clinic. We have limited control over the nature or timing of their research and limited visibility into their day-to-day activities, and as a result can provide little assurance that their efforts will be successful.

We contract with third parties for the manufacture of our product candidates for preclinical and clinical testing and may also do so for commercialization, if and when our product candidates are approved. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or any future product candidate or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We may rely on third-party manufacturers or third-party collaborators for the manufacture of commercial supply of one or more product candidates for which our collaborators or we obtain marketing approval. We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including, but not necessarily limited to:

- reliance on the third party for regulatory compliance and quality assurance, while still being required by law to establish adequate oversight and control over products furnished by that third party;
- the possible breach of the manufacturing agreement by the third party;
- manufacturing delays if our third-party manufacturers are unable to obtain raw materials due to supply chain disruptions, give greater priority to the supply of other products over our product candidates or otherwise do not satisfactorily perform according to the terms of the agreement between us;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

We rely on our third-party manufacturers to produce or purchase from third-party suppliers the materials and equipment necessary to produce our product candidates for our preclinical and clinical trials. Forces beyond our control, including the effects of the COVID-19 pandemic, could disrupt the global supply chain and impact our or our third-party manufacturers' ability to obtain raw materials or other products necessary to manufacture our product candidates. There are a limited number of suppliers for raw materials and equipment that we use (or that are used on our behalf) to manufacture our drugs, and there may be a need to assess alternate suppliers to prevent a possible disruption of the manufacture of the materials and equipment necessary to produce our product candidates for our preclinical and clinical trials, and if approved, ultimately for commercial sale. We do not have any control over the process or timing of the acquisition of these raw materials or equipment by our third-party manufacturers. Any significant delay in the supply of a product candidate, or the raw material components thereof, for an ongoing preclinical or clinical trial due to the need to replace a third-party manufacturer could considerably delay completion of our preclinical or clinical trials, product testing and potential regulatory approval of our product candidates. If our manufacturers or we are unable to purchase these raw materials or equipment after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of our product candidates.

The facilities used by contract manufacturers to potentially manufacture our product candidates must be approved by the FDA pursuant to inspections that will be conducted after we submit an NDA or BLA to the FDA. We are required by law to establish adequate oversight and control over raw materials, components and finished products furnished by our contract manufacturers, but we do not control the day-to-day manufacturing operations of, and are dependent on, the contract manufacturers for compliance with cGMP regulations for manufacture of our product candidates. Third-party manufacturers may not be able to comply with the cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, restrictions on imports and exports, civil penalties, delays, suspension or withdrawal of approvals, license revocation, 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.

One or more of the product candidates that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply. If our current contract manufacturers cannot perform as agreed, we may be required to replace such manufacturers. We may incur added costs and delays in identifying and qualifying any replacement manufacturers. The DEA restricts the importation of a controlled substance finished drug product when the same substance is commercially available in the United States, which could reduce the number of potential alternative manufacturers for one or more of our product candidates.

Future dependence upon others for the manufacture of our product candidates or products may adversely affect our future profit margins and our ability to commercialize any products that may receive marketing approval on a timely and competitive basis.

We also expect to rely on other third parties to 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, if approved, producing additional losses and depriving us of potential product revenue.

We rely on third parties to conduct all aspects of our lentiviral vector production and these third parties may not perform satisfactorily.

We do not independently conduct our lentiviral vector production and we currently rely, and expect to continue to rely, on third parties with respect to the manufacture of these items.

Our reliance on these third parties for manufacturing lentiviral vector reduces our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For products that we develop and commercialize, we will remain responsible for ensuring that each of our IND-enabling studies and clinical studies is conducted in accordance with the study plan and protocols, and that our lentiviral vectors are manufactured in accordance with GMP as applied in the relevant jurisdictions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines, conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, or manufacture our lentiviral vectors in accordance with GMP, we will not be able to complete, or may be delayed in completing, the preclinical and clinical studies and manufacturing process validation activities required to support future IND, market authorization application and BLA submissions and approval of our product candidates, or to support commercialization of our products, if approved. Many of our agreements with these third parties contain termination provisions that allow these third parties to terminate their relationships with us at any time. If we need to enter into alternative arrangements, our product development and commercialization activities could be delayed.

We may be forced to enter into an agreement with a different manufacturer, which we may not be able to do on reasonable terms, if at all. In some cases, the technical skills required to manufacture lentiviral vector for our drug product candidates may be unique or proprietary to the original manufacturer, and we may have difficulty or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternate supplier, or we may be unable to transfer such skills at all. Any of these events could lead to clinical study delays or failure to obtain marketing approval or impact our ability to successfully commercialize our product or any future products. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of production.

We rely on clinical data and results obtained by third parties that could ultimately prove to be inaccurate or unreliable.

As part of our strategy to mitigate development risk, we seek to develop product candidates with well-studied mechanisms of action, and we utilize biomarkers to assess potential clinical efficacy early in the development process. This strategy necessarily relies upon clinical data and other results obtained by third parties that may ultimately prove to be inaccurate or unreliable. Further, such clinical data and results

may be based on products or product candidates that are significantly different from our product candidates or any future product candidate. If the third-party data and results we rely upon prove to be inaccurate, unreliable or not applicable to our product candidates or future product candidate, we could make inaccurate assumptions and conclusions about our product candidates and our research and development efforts could be compromised.

We may need to license certain intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property, including patent rights that are important or necessary to the development and commercialization of our products. It may be necessary for us to use the patented or proprietary technology of third parties, who may or may not be interested in granting such a license, to commercialize our products, in which case we would be required to obtain a license from these third parties on commercially reasonable terms, or our business could be harmed, possibly materially.

Collaborative relationships with third parties could cause us to expend significant resources and incur substantial business risk with no assurance of financial return.

Establishing strategic collaborations is difficult and time-consuming. Our discussions with potential collaborators may not lead to the establishment of collaborations on favorable terms, if at all. Potential collaborators may reject collaborations based upon their assessment of our financial, regulatory or intellectual property position. In addition, there has been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. Even if we successfully establish new collaborations, these relationships may never result in the successful development or commercialization of product candidates or the generation of sales revenue. To the extent that we enter into collaborative arrangements, the related product revenues are likely to be lower than if we directly marketed and sold products. Such collaborators may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for any future product candidate.

Risks Relating to Legislation and Regulation Affecting the Biopharmaceutical and Other Industries

We are subject to new legislation, regulatory proposals and managed care initiatives that may increase our costs of compliance and adversely affect our ability to market our products, obtain collaborators and raise capital.

Legislative and regulatory changes to the healthcare systems of the United States and certain foreign countries could impact our ability to sell our products profitably. In particular, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 ("MMA") changed the way Medicare covers and pays for pharmaceutical products by revising the payment methodology for many products reimbursed by Medicare, resulting in lower rates of reimbursement for many types of drugs, and added a prescription drug benefit to the Medicare program that involves commercial plans negotiating drug prices for their members. In addition, this law provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this law and future laws could decrease the coverage and price that we will receive for any approved products. While the MMA only applies to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Therefore, any limitations in reimbursement that results from the MMA may result in reductions in payments from private payors.

Since 2003, there have been several other legislative and regulatory changes to the coverage and reimbursement landscape for pharmaceuticals. In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, collectively, the "Affordable Care Act" or "ACA," was enacted in 2010 and made significant changes to the United States' healthcare system. The ACA and any revisions or replacements of that Act, any substitute legislation, and other changes in the law or regulatory framework could have a material adverse effect on our business.

Among the provisions of the ACA of importance to our potential product candidates are:

- an annual, nondeductible fee on any entity that manufactures, or imports specified branded prescription drugs and biological products, apportioned among these entities according to their market share in certain government healthcare programs;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13.0% of the average manufacturer price for branded and generic drugs, respectively;

- expansion of healthcare fraud and abuse laws, including the federal False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for non-compliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for a manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of a manufacturer's Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for certain individuals with income at or below 138% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;
- expansion of the entities eligible to enroll in the 340B Drug Pricing Program to include certain critical access hospitals, freestanding cancer hospitals, rural referral centers, and sole community hospitals, but exempting certain drugs from the ceiling price requirements for these covered entities;
- the new requirements under the federal Open Payments program and its implementing regulations;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians;
- a new regulatory pathway for the approval of biosimilar biological products, all of which will impact existing government healthcare programs and will result in the development of new programs; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

The Supreme Court upheld the ACA in the main challenge to the constitutionality of the law in 2012. Specifically, the Supreme Court held that the individual mandate and corresponding penalty was constitutional because it would be considered a tax by the federal government. The Supreme Court also upheld federal subsidies for purchasers of insurance through federally facilitated exchanges in a decision released in June 2015.

At the end of 2017, Congress passed the Tax Cuts and Jobs Act, which repealed the penalty for individuals who fail to maintain minimum essential health coverage as required by the ACA. Following this legislation, Texas and 19 other states filed a lawsuit alleging that the ACA is unconstitutional as the individual mandate was repealed, undermining the legal basis for the Supreme Court's prior decision. On December 14, 2018, a Texas federal district court judge issued a ruling declaring that the ACA in its entirety is unconstitutional. Upon appeal, the Fifth Circuit upheld the district court's ruling that the individual mandate is unconstitutional. However, the Fifth Circuit remanded the case back to the district court to conduct a more thorough assessment of the constitutionality of the entire ACA despite the individual mandate being unconstitutional. The Supreme Court agreed to hear the case on appeal from the Fifth Circuit on March 2, 2020 and held oral arguments on November 10, 2020. While this lawsuit has no immediate legal effect on the ACA and its provisions, this lawsuit is ongoing and the outcome may have a significant impact on our business.

The Bipartisan Budget Act of 2018, the "BBA," which set government spending levels for Fiscal Years 2018 and 2019, revised certain provisions of the ACA. Specifically, beginning in 2019, the BBA increased manufacturer point-of-sale discounts off negotiated prices of applicable brand drugs in the Medicare Part D coverage gap from 50% to 70%, ultimately increasing the liability for brand drug manufacturers. Further, this mandatory manufacturer discount applied to biosimilars beginning in 2019.

The 116th Congress explored legislation intended to address the cost of prescription drugs. Notably, the major committees of jurisdiction in the Senate (Finance Committee, Health, Education, Labor and Pensions Committee, and Judiciary Committee), marked up legislation intended to address various elements of the prescription drug supply chain. Proposals include a significant overhaul of the Medicare Part D benefit design, addressing patent "loopholes", and efforts to cap the increase in drug prices. The House Energy and Commerce Committee approved drug-related legislation intended to increase transparency of drug prices and also curb anti-competitive behavior in the pharmaceutical supply chain. In addition, the House Ways & Means Committee approved legislation intended to improve drug price transparency, including for drug manufacturers to justify certain price increases. The 117th Congress convened on January 3, 2021, and could reintroduce many of the bills targeting drug prices. While we cannot predict what proposals may ultimately become law, the elements under consideration could significantly change the landscape in which the pharmaceutical market operates.

The Senate Committee on Health, Education, Labor, and Pensions (HELP) advanced the Lower Health Care Costs Act of 2019. Among other things, the bill is intended to reduce costs in the United States health sector. The bill revises certain requirements to expedite the approval of generics and biosimilars. It also limits prices that pharmacy benefit managers may charge health insurers or enrollees for prescription drugs. Although this bill still needs to pass the full Senate and House of Representatives, it is worth noting the wide-ranging effects it could have on the health care sector.

On December 12, 2019, the House of Representatives passed broad legislation (H.R. 3, the *Elijah E. Cummings Lower Drug Costs Now Act*) that would, among other provisions, require the Department of Health and Human Services ("HHS") to negotiate drug prices and impose price caps and restructure the Medicare Part D benefit, imposing more financial responsibility on certain drug manufacturers. Failure by a manufacturer to reach an agreement with HHS on the negotiated price could result in significant penalties for prescription drug manufacturers. In addition, S. 2543, *the Prescription Drug Pricing Reduction Act* would also, among other provisions, restructure the Medicare Part D benefit, but it would not authorize direct negotiation by the federal government. While we cannot predict what proposals may ultimately become law, the elements under consideration could significantly change the landscape in which the pharmaceutical market operates.

The Trump Administration took several regulatory steps to redirect ACA implementation. The HHS finalized a Medicare hospital payment reduction for Part B drugs acquired through the 340B Drug Pricing Program. Under the Trump Administration, HHS finalized several proposals aimed at lowering drug prices for Medicare beneficiaries and increasing price transparency. For example, the Trump Administration issued an interim final rule on November 27, 2020 implementing a "Most Favored Nation" payment model for Part B drugs that applies international reference pricing to determine reimbursement for certain drugs paid by Medicare Part B. The interim final rule was enjoined by federal courts prior to its implementation date of January 1, 2021, and the lawsuit is ongoing. In addition, HHS, in conjunction with the FDA, finalized four pharmaceutical importation pathways in September 2020: (1) regulations establishing importation of pharmaceuticals from Canada by wholesalers and pharmacists; (2) FDA guidance permitting manufacturers to import their own pharmaceuticals that were originally intended for marketing in other countries; (3) a request for proposals from private sector entities to import prescription drugs for personal use under existing statutory authority; and (4) a request for proposals from private sector entities to reimport insulin under existing statutory authority. Further, on November 11, 2020, the Trump Administration issued a final rule that changes the permissible structure of drug rebates and discounts between drug manufacturers and third-party payors (including pharmacy benefit managers that negotiate drug prices on behalf of such third-party payors). This final rule, often referred to as the "Rebate Rule," could have significant direct and indirect impacts on drug pricing in both government and commercial markets. With respect to price transparency, the Trump Administration promulgated regulations that require hospitals and third-party payors to disclose prices of items and services, which may impact negotiated rates in the commercial market.

On January 20, 2021, Joe Biden was inaugurated as the 46th president of the United States. As a presidential candidate, Mr. Biden indicated support for several policies aimed at lowering drug prices, including government price negotiation, drug importation, international reference pricing, and price increase controls. The incoming Biden Administration may continue, modify, or repeal many of the drug pricing policies proposed and finalized by the Trump Administration. While we cannot predict which policies the Biden Administration may support and enforce, the policies finalized in the months prior to the beginning of Mr. Biden's term, if continued, could significantly change the landscape in which the pharmaceutical market operates and significantly impact our ability to effectively market and sell our products.

There likely will continue to be legislative and regulatory proposals at the federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare products and services. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare may adversely affect:

- the demand for any products for which we may obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to generate revenues and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

In addition, governments may impose price controls, which may adversely affect our future profitability. In January 2020, President Trump signed into law the U.S.-Mexico-Canada (USMCA) trade deal into law. As enacted, there are no commitments with respect to biological product intellectual property rights or data protection, which may create an unfavorable environment across these three countries.

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

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

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.

We cannot predict the likelihood, nature or extent of how government regulation that may arise from future legislation or administrative or executive action taken by the U.S. presidential administration may impact our business and industry. In particular, the prior Administration took several executive actions, specifically through rulemaking and guidance, that could impact the pharmaceutical business and industry. Shortly after taking office in January 2021, President Biden announced that his Administration would be freezing a number of the prior Administration's drug pricing reforms, while others remain subject to both executive orders or regulatory changes issued by the Department of Health and Human Services. A few of the major administrative actions include:

- On October 30, 2019, the Trump Administration issued an advanced notice of proposed rulemaking ("ANPRM") entitled, *International Pricing Index Model for Medicare Part B Drugs*. This ANPRM was intended to solicit feedback on a potential proposal to align United States drug prices in the Medicare Part B program with international prices. It also solicited public feedback on a policy that would allowing private-sector vendors to negotiate prices, take title to drugs, and improve competition for hospital and physician business. Although this is only a notice for a potential rule, it signals the Administration's desire to regulatorily influence the United States drug pricing system that could adversely affect the industry.

- On November 15, 2019, CMS issued a proposed rule entitled, *Transparency in Coverage* and finalized the *Calendar Year ("CY") 2020 Outpatient Prospective Payment System ("OPPS") & Ambulatory Surgical Center Price Transparency Requirements for Hospitals to Make Standard Charges Rule*. Together the rules would increase price transparency through health plans and in hospitals. The affects may influence consumer purchasing habits in the health care sector as a whole. Although the transparency provisions are not yet in effect and the hospital price transparency requirements are subject to litigation, there could be implications for the industry related to drug pricing if or when it is enacted.

- On November 18, 2019, CMS issued a proposed rule entitled, *Medicaid Fiscal Accountability Regulation ("MFAR")*. The proposed rule would significantly impact states' ability to finance their Medicaid programs. If finalized, the MFAR could force states to restructure their Medicaid financing that could disincentivize or change state prescription drug purchasing behavior that would adversely impact the industry.

- On December 18, 2019, the FDA issued a proposed rule entitled, *Importation of Prescription Drugs.* The proposed rule would allow the importation of certain prescription drugs from Canada. If finalized, states or other non-federal government entities would be able to submit importation program proposals to FDA for review and authorization. This proposed rule could also influence pricing practices in the United States.

- On January 30, 2020, CMS issued a state waiver option entitled, *Health Adult Opportunity ("HAO")*. The HAO would allow states to restructure benefits and coverage policies for their Medicaid programs. The HAO will provide states administrative flexibilities in exchange for a capped federal share. The cap on the federal share is commonly referred to as a "block grant." Importantly, the HAO allows states to set formularies that align with Essential Health Benefit requirements while still requiring manufacturers to participate in the Medicaid Rebate Program. Depending on utilization of the HAO by states, it could impact the industry – especially if states elect to use a formulary.

- On December 2, 2020, CMS issued a final rule entitled, *Modernizing and Clarifying the Physician Self-Referral Regulations* and on the same day the HHS Office of Inspector General finalized a similar rule, entitled *Revisions to Safe Harbors Under the Anti-Kickback Statute, and Civil Monetary penalty Rules Regarding Beneficiary Inducements*. The rules are an effort to reform regulations dealing with anti-kickback and self-referral laws. These rules allow certain financial arrangements that would otherwise violate anti-kickback and self-referral laws for providers that are participating in value-based payment arrangements. The rule could impact drug purchasing behavior to ensure providers are within their budget and/or restructure existing payment structures between providers and manufacturers.

As with any change in the Executive Office, and particularly with respect to changes from a Republican Administration under former President Trump to a Democratic Administration under President Biden, we expect there to be significant changes to existing rules, regulations and policies, the enactment of new Executive Orders and other immediate or iterative political, legislative and administrative changes, affecting the pharmaceutical industry. 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 based on similar governmental changes in other countries.

Changes in funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business or the business of our partners.

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, ability to hire and retain key personnel, ability to accept the payment of user fees, and statutory, regulatory, and policy changes. 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 drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business or the business of our partners. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough nonessential FDA employees and stop routine activities. If 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. If the timing of FDA's review and approval of new products is delayed, the timing of our or our partners' development process may be delayed, which could result in delayed milestone revenues and materially harm our operations or business.

The COVID-19 pandemic caused considerable disruptions at the FDA, namely with respect to diverting the FDA's attention and resources to facilitate vaccine development and ensure rapid review and emergency use authorization of vaccines intended to prevent COVID-19. The enhanced focus on COVID-19 countermeasures, and the reorganization and rededication of critical resources, both at the FDA and within similar governmental authorities across the world, impact the ability of new products and services from being developed or commercialized in a timely manner.

Our current and future relationships with customers and third-party payors in the United States and elsewhere may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the U.S. and elsewhere will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, which may constrain the business or financial arrangements and relationships through which we sell, market and distribute any product candidates for which we obtain marketing approval. In addition, we may be subject to transparency laws and patient privacy regulation by the federal and state governments and by governments in foreign jurisdictions in which we conduct our business. The applicable federal, state and foreign healthcare laws and regulations that may affect our ability to operate include, but are not necessarily limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs, such as Medicare and Medicaid;

- federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government; the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 ("HITECH"), and their respective implementing regulations, which impose obligations on covered healthcare providers, health plans, and healthcare clearinghouses, as well as their business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

- the federal Open Payments program, which requires manufacturers of certain approved drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services ("CMS"), information related to "payments or other transfers of value" made to physicians, which is defined to include doctors, dentists, optometrists, podiatrists and chiropractors, and teaching hospitals and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by the physicians and their immediate family members. Data collection began on August 1, 2013, with requirements for manufacturers to submit reports to CMS by March 31, 2014, and 90 days after the end each subsequent calendar year. Disclosure of such information was made by CMS on a publicly available website beginning in September 2014 and is annually updated; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, including, without limitation, damages, fines, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations, which could have a material adverse effect on our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business, including our collaborators, is found not to be in compliance with applicable laws, it may be subject to criminal, civil or administrative sanctions, including exclusions from participation in government healthcare programs, which could also materially affect our business.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. Although we believe that the safety procedures for handling and disposing of these materials comply with the standards prescribed by these laws and regulations, we cannot eliminate the risk of accidental 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. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

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

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

Risks Related to Intellectual Property and Potential Disputes Thereof

If we are unable to obtain and maintain sufficient patent protection for our technology and products, our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology and products may be impaired.

Our success depends, in large part, on our ability to obtain patent protection for product candidates and their formulations and uses. The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or our partners will be successful in obtaining patents or what the scope of an issued patent may ultimately be. These risks and uncertainties include, but are not necessarily limited to, the following:

- patent applications may not result in any patents being issued, or the scope of issued patents may not extend to competitive product candidates and their formulations and uses developed or produced by others;
- our competitors, many of which have substantially greater resources than us or our partners, and many of which have made significant investments in competing technologies, may seek, or may already have obtained, patents that may limit or interfere with our abilities to make, use, and sell potential product candidates, file new patent applications, or may affect any pending patent applications that we may have;
- there may be significant pressure on the U.S. government and other 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 products.

In addition, 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. Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO, or become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. The costs of these proceedings could be substantial, and it is possible that our efforts to establish priority of invention would be unsuccessful, resulting in a material adverse effect on our U.S. patent positions. An adverse determination in any such submission, patent office trial, proceeding or litigation could reduce the scope of, render unenforceable, or invalidate, our patent rights, allow third parties to commercialize our technologies or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates. Third parties are often responsible for maintaining patent protection for our product candidates, at our and their expense. If that party fails to appropriately prosecute and maintain patent protection for a product candidate, our abilities to develop and commercialize products may be adversely affected, and we may not be able to prevent competitors from making, using and selling competing products. Such a failure to properly protect intellectual property rights relating to any of our product candidates could have a material adverse effect on our financial condition and results of operations. In addition, U.S. patent laws may change, which could prevent or limit us from filing patent applications or patent claims to protect products and/or technologies or limit the exclusivity periods that are available to patent holders, as well as affect the validity, enforceability, or scope of issued patents.

We and our licensors also rely on trade secrets and proprietary know-how to protect product candidates. Although we have taken steps to protect our and their trade secrets and unpatented know-how, including entering into confidentiality and non-use agreements with third parties, and proprietary information and invention assignment agreements with employees, consultants and advisers, third parties may still

come upon this same or similar information independently. Despite these efforts, any of these parties may also breach the agreements and may unintentionally or willfully disclose our or our licensors' proprietary information, including our trade secrets, and we may not be able to identify such breaches or obtain adequate remedies. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Moreover, if any of our or our licensors' trade secrets were to be lawfully obtained or independently developed by a competitor, we and our licensors 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 any of our or our licensors' trade secrets were to be disclosed to or independently developed by a competitor, our competitive positions would be harmed.

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify any patentable aspects of our research and development output and methodology, and, even if we do, an opportunity to obtain patent protection may have passed. Given the uncertain and time-consuming process of filing patent applications and prosecuting them, it is possible that our product(s) or process(es) originally covered by the scope of the patent application may have changed or been modified, leaving our product(s) or process(es) without patent protection. If our licensors or we fail to obtain or maintain patent protection or trade secret protection for one or more product candidates or any future product candidate we may license or acquire, third parties may be able to leverage our proprietary information and products without risk of infringement, which could impair our ability to compete in the market and adversely affect our ability to generate revenues and achieve profitability. Moreover, should we enter into other collaborations we may be required to consult with or cede control to collaborators regarding the prosecution, maintenance and enforcement of licensed patents. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, no consistent policy regarding the breadth of claims allowed in pharmaceutical or biotechnology patents has emerged to date in the U.S. The patent situation outside the U.S. is even more uncertain. The laws of foreign countries may not protect our rights to the same extent as the laws of the U.S., and we may fail to seek or obtain patent protection in all major markets. For example, European patent law restricts the patentability of methods of treatment of the human body more than U.S. law does. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after a first filing, or in some cases not at all. Therefore, we cannot know with certainty whether we or our licensors were the first to make the inventions claimed in patents or pending patent applications that we own or licensed, or that we or our licensors were the first to file for patent protection of such inventions. In the event that a third party has also filed a U.S. patent application relating to our product candidates or a similar invention, depending upon the priority dates claimed by the competing parties, we may have to participate in interference proceedings declared by the USPTO to determine priority of invention in the U.S. We might also become involved in derivation proceedings in an event that a third party misappropriates one or more of our inventions and files their own patent application directed to such one or more inventions. The costs of these proceedings could be substantial, and it is possible that our efforts to establish priority of invention (or that a third party derived an invention from us) would be unsuccessful, resulting in a material adverse effect on our U.S. patent position. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the U.S. and other countries may diminish the value of our patents or narrow the scope of our patent protection. For example, the federal courts of the U.S. have taken an increasingly dim view of the patent eligibility of certain subject matter, such as naturally occurring nucleic acid sequences, amino acid sequences and certain methods of utilizing the same, which include their detection in a biological sample and diagnostic conclusions arising from their detection. Such subject matter, which had long been a staple of the biotechnology and biopharmaceutical industry to protect their discoveries, is now considered, with few exceptions, ineligible in the first instance for protection under the patent laws of the U.S. Accordingly, we cannot predict the breadth of claims that may be allowed and remain enforceable in our patents or in those licensed from a third party.

Even if our patent applications issue as patents, they may not issue in a form that will 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 owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

We also may rely on the regulatory period of market exclusivity for any of our biologic product candidates that are successfully developed and approved for commercialization. Although this period in the United States is generally 12 years from the date of marketing approval (depending on the nature of the specific product), there is a risk that the U.S. Congress could amend laws to significantly shorten this exclusivity period. Once any regulatory period of exclusivity expires, depending on the status of our patent coverage and the nature of the product, we may not be able to prevent others from marketing products that are biosimilar to or interchangeable with our products, which would materially adversely affect our business.

We depend on our licensors to maintain and enforce the intellectual property covering certain of our product candidates. We have limited, if any, control over the resources that our licensors can or will devote to securing, maintaining, and enforcing patents protecting our product candidates.

We depend on our licensors to protect the proprietary rights covering our product candidates and we have limited, if any, control over the amount or timing of resources that they devote on our behalf, or the priority they place on, maintaining patent rights and prosecuting patent applications to our advantage. Moreover, we have limited, if any, control over the strategies and arguments employed in the maintenance of patent rights and the prosecution of patent applications to our advantage. Our licensors might become involved in disputes with one of their other licensees, and we or a portion of our licensed patent rights might become embroiled in such disputes.

Our licensors, depending on the patent or application, are responsible for maintaining issued patents and prosecuting patent applications. We cannot be sure that they will perform as required. Should they decide they no longer want to maintain any of the patents licensed to us, they are required to afford us the opportunity to do so at our expense. If our licensors do not perform, and if we do not assume the maintenance of the licensed patents in sufficient time to make required payments or filings with the appropriate governmental agencies, we risk losing the benefit of all or some of those patent rights. Moreover, and possibly unbeknownst to us, our licensors may experience serious difficulties related to their overall business or financial stability, and they may be unwilling or unable to continue to expend the financial resources required to maintain and prosecute these patents and patent applications. While we intend to take actions reasonably necessary to enforce our patent rights, we depend, in part, on our licensors to protect a substantial portion of our proprietary rights and to inform us of the status of those protections and efforts thereto.

Our licensors may also be notified of alleged infringement and be sued for infringement of third-party patents or other proprietary rights. We may have limited, if any, control or involvement over the defense of these claims, and our licensors could be subject to injunctions and temporary or permanent exclusionary orders in the U.S. or other countries. Our licensors are not obligated to defend or assist in our defense against third-party claims of infringement. We have limited, if any, control over the amount or timing of resources, if any, that our licensors devote on our behalf or the priority they place on defense of such third-party claims of infringement.

Because of the uncertainty inherent in any patent or other litigation involving proprietary rights, we or our licensors may not be successful in defending claims of intellectual property infringement alleged by third parties, which could have a material adverse effect on our results of operations. Regardless of the outcome of any litigation, defending the litigation may be expensive, time-consuming and distracting to management.

Protecting our proprietary rights is difficult and costly, and we may be unable to ensure their protection.

The degree of future protection for our proprietary rights is uncertain, because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage, in addition to being costly and time consuming to undertake. For example:

- our licensors might not have been the first to make the inventions covered by each of our pending patent applications and issued patents;
- our licensors might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate our product candidates or any future product candidate technologies;
- it is possible that none of the pending patent applications licensed to us will result in issued patents;
- the scope of our issued patents may not extend to competitive products developed or produced by others;
- the issued patents covering our product candidates or any future product candidate may not provide a basis for market exclusivity for active products, may not provide us with any competitive advantages, or may be challenged by third parties;
- we may not develop additional proprietary technologies that are patentable; or
- intellectual property rights of others may have an adverse effect on our business.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful, and an unfavorable outcome in any litigation would harm our business.

Competitors may infringe our issued patents or other intellectual property. To counter infringement or unauthorized use, we may be required to file one or more actions for patent infringement, which can be expensive and time consuming. Any claims we assert against accused infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents; or provoke those parties to petition the USPTO to institute *inter partes* review against the asserted patents, which may lead to a finding that all or some of the claims of the patent are invalid. In addition, in a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question or as a matter of public policy. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, rendered unenforceable, or interpreted narrowly. Furthermore, adverse results on U.S. patents may affect related patents in our global portfolio.

If we or our licensors are sued for infringing intellectual property rights of third parties, it will be costly and time consuming, and an unfavorable outcome in that litigation would have a material adverse effect on our business.

Our success also depends on our ability, and the abilities of any of our respective current or future collaborators, to develop, manufacture, market and sell product candidates without infringing the proprietary rights of third parties. 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 products, some of which may be directed at claims that overlap with the subject matter of our or our licensors' intellectual property. Because patent applications can take many years to issue, there may be currently pending applications, unknown to us, which may later result in issued patents that our product candidates or proprietary technologies may infringe. Similarly, there may be issued patents relevant to our product candidates of which we or our licensors are not aware. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after a first filing, or in some cases not at all. Therefore, we cannot know with certainty whether we or such licensors were the first to make the inventions claimed in patents or pending patent applications that we own or licensed, or that we and our licensors were the first to file for patent protection of such inventions. In the event that a third party has also filed a U.S. patent application relating to our product candidates or a similar invention, depending upon the priority dates claimed by the competing parties, we may have to participate in interference proceedings declared by the USPTO to determine priority of invention in the U.S. The costs of these proceedings could be substantial, and it is possible that our efforts to establish priority of invention would be unsuccessful, resulting in a material adverse effect on our U.S. patent position. As a result, the issuance, scope, validity, enforceability and commercial value of our or any of our licensors' patent rights are highly uncertain.

There is a substantial amount of litigation involving patent and other intellectual property rights in the biotechnology and biopharmaceutical industries generally. If a third party claims that we or any of our licensors, suppliers or collaborators infringe the third party's intellectual property rights, we may have to, among other things:

- obtain additional licenses, which may not be available on commercially reasonable terms, if at all;
- abandon an infringing product candidate or redesign products or processes to avoid infringement, which may demand substantial funds, time and resources and which may result in inferior or less desirable processes and/or products;
- pay substantial damages, including the possibility of treble damages and attorneys' fees, if a court decides that the product or proprietary technology at issue infringes on or violates the third party's rights;
- pay substantial royalties, fees and/or grant cross-licenses to our product candidates; and/or
- defend litigation or administrative proceedings which may be costly regardless of outcome, and which could result in a substantial diversion of financial and management resources.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings

adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

If we fail to comply with our obligations under our intellectual property licenses and third party funding arrangements, we could lose rights that are important to our business.

We are currently a party to license agreements with St. Jude, COH, Fred Hutch, UCLA, Nationwide and other institutions. In the future, we may become party to licenses that are important for product development and commercialization. If we fail to comply with our obligations under current or future license and funding agreements, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market any product or utilize any technology that is covered by these agreements or may face other penalties under the agreements. Such an occurrence could materially and adversely affect the value of a product candidate being developed under any such agreement or could restrict our drug discovery activities. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

We may be subject to claims that our employees and/or consultants have wrongfully used or disclosed to us alleged trade secrets of their former employers or other clients.

As is common in the biopharmaceutical industry, we rely on employees and consultants to assist in the development of product candidates, many of whom were previously employed at, or may have previously been or are currently providing consulting services to, other biopharmaceutical companies, including our competitors or potential competitors. We may become subject to claims related to whether these individuals have inadvertently or otherwise used, disclosed or misappropriated trade secrets or other proprietary information of their former employers or their former or current clients. Litigation may be necessary to defend against these claims. Even if we are successful in defending these claims, litigation could result in substantial costs and be a distraction to management and/or the employees or consultants that are implicated.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patent protection for our product candidates or any future product candidate, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position, particularly where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We limit disclosure of such trade secrets where possible but we also seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who do have access to them, such as our employees, our licensors, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and may unintentionally or willfully disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Moreover, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, 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 any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

We in-license intellectual property pertaining to certain product candidates from third parties. As such, any dispute with the licensors or the non-performance of such license agreements may adversely affect our ability to develop and commercialize the applicable product candidates.

The types of disputes which may arise between us and the third parties from whom we license intellectual property include, but are not limited to:

- the scope of rights granted under such license agreements and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to such license agreements;

- the scope and interpretation of the representations and warranties made to us by our licensors, including those pertaining to the licensors' right title and interest in the licensed technology and the licensors' right to grant the licenses contemplated by such agreements;
- the sublicensing of patent and other rights under our license agreements and/or collaborative development relationships, and the rights and obligations associated with such sublicensing, including whether or not a given transaction constitutes a sublicense under such license agreement;
- the diligence and development obligations under license agreements (which may include specific diligence milestones) and what activities or achievements satisfy those diligence obligations;
- whether or not the milestones associated with certain milestone payment obligations have been achieved or satisfied;
- the applicability or scope of indemnification claims or obligations under such license agreements;
- the permissibility and advisability of, and strategy regarding, the pursuit of potential third-party infringers of the intellectual property that is the subject of such license agreements;
- the calculation of royalty, sublicense revenue and other payment obligations under such license agreements;
- the extent to which license rights, if any, are retained by licensors under such license agreements;
- whether or not a material breach has occurred under such license agreements and the extent to which such breach, if deemed to have occurred, is or can be cured within applicable cure periods, if any;
- disputes regarding patent filing and prosecution decisions, as well as payment obligations regarding past and ongoing patent expenses;
- intellectual property rights resulting from the joint creation or use of intellectual property (including improvements made to licensed intellectual property) by our and our partners' licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations or may conflict in such a way that puts us in breach of one or more agreements, which would make us susceptible to lengthy and expensive disputes with one or more of such third-party licensing partners. 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 agreements, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations and prospects.

Risks Relating to Our Control by Fortress Biotech Inc.

Fortress controls a voting majority of our common stock.

Pursuant to the terms of the Class A Preferred Stock held by Fortress, Fortress is entitled to cast, for each share of Class A Preferred held by Fortress, the number of votes that is equal to one and one-tenth (1.1) times a fraction, the numerator of which is the sum of (A) the shares of outstanding common stock and (B) the whole shares of common stock into which the shares of outstanding Class A common shares and the Class A Preferred Stock are convertible and the denominator of which is the number of shares of outstanding Class A Preferred Stock. Accordingly, Fortress is able to control or significantly influence all matters requiring approval by our stockholders, including the election of directors and the approval of mergers or other business combination transactions. The interests of Fortress may not always coincide with the interests of other stockholders, and Fortress may take actions that advance its own interests and are contrary to the desires of our other stockholders. Moreover, this concentration of voting power may delay, prevent or deter a change in control of us even when such a change may be in the best interests of all stockholders, could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of Mustang or our assets, and might affect the prevailing market price of our common stock.

Fortress has the right to receive a significant grant of shares of our common stock annually which will result in the dilution of your holdings of common stock upon each grant, which could reduce their value.

Under the terms of the Second Amended and Restated Founders Agreement, which became effective July 22, 2016, Fortress will receive a grant of shares of our common stock equal to two and one-half percent (2.5%) of the gross amount of any equity or debt financing. Additionally, the Class A Preferred Stock, as a class, will receive an annual dividend on January 1st, payable in shares of common stock in an amount equal to two and one-half percent (2.5%) of our fully-diluted outstanding capital stock as of the business day immediately prior to January 1st of such year. Fortress currently owns all outstanding shares of Class A Preferred Stock. These share issuances to Fortress and any other holder of Class A Preferred Stock will dilute your holdings in our common stock and, if the value of Mustang has not grown proportionately over the prior year, would result in a reduction in the value of your shares. The Second Amended and Restated Founders Agreement has a term of 15 years and renews automatically for subsequent one-year periods unless terminated by Fortress or upon a Change in Control (as defined in the Second Amended and Restated Founders Agreement).

We might have received better terms from unaffiliated third parties than the terms we receive in our agreements with Fortress.

The agreements we have entered into with Fortress include a Management Services Agreement and the Founders Agreement. While we believe the terms of these agreements are reasonable, they might not reflect terms that would have resulted from arm's-length negotiations between unaffiliated third parties. The terms of the agreements relate to, among other things, payment of a royalty on product sales and the provision of employment and transition services. We might have received better terms from third parties because, among other things, third parties might have competed with each other to win our business.

The dual roles of our directors who also serve in similar roles with Fortress could create a conflict of interest and will require careful monitoring by our independent directors.

We share some directors with Fortress which could create conflicts of interest between the two companies in the future. While we believe that the Founders Agreement and the Management Services Agreement were negotiated by independent parties on both sides on arm's length terms, and the fiduciary duties of both parties were thereby satisfied, in the future situations may arise under the operation of both agreements that may create a conflict of interest. We will have to be diligent to ensure that any such situation is resolved by independent parties. In particular, under the Management Services Agreement, Fortress and its affiliates are free to pursue opportunities which could potentially be of interest to Mustang, and they are not required to notify Mustang prior to pursuing such opportunities. Any such conflict of interest or pursuit by Fortress of a corporate opportunity independent of Mustang could expose us to claims by our investors and creditors and could harm our results of operations.

General Risks

Major public health issues, and specifically the pandemic caused by the spread of COVID-19, could have an adverse impact on our financial condition and results of operations and other aspects of our business.

In December 2019, a novel strain of coronavirus, COVID-19, was first detected in Wuhan, China, and has since spread around the world. On March 11, 2020, the World Health Organization declared that the rapidly spreading COVID-19 outbreak had evolved into a pandemic. In response to the pandemic, many governments around the world implemented a variety of measures to reduce the spread of COVID-19, including travel restrictions and bans, instructions to residents to practice social distancing, quarantine advisories, shelter-in-place orders and required closures of non-essential businesses.

The COVID-19 pandemic negatively impacted the global economy, disrupted global supply chains, and created significant volatility and disruption of financial markets. Although COVID-19 has not had a material adverse effect on our business to date, no assurance can be given that it will not in the future if the situation persists or worsens. The extent to which the coronavirus impacts our business and operating results will depend on future developments that are highly uncertain and cannot be accurately predicted, including new information that may emerge concerning the coronavirus and the actions to contain the coronavirus or treat its impact, among others.

Should the coronavirus continue to spread, our business operations could be delayed or interrupted. For instance, our clinical trials may be affected by the pandemic. Site initiation, participant recruitment and enrollment, participant dosing, distribution of clinical trial materials, study monitoring and data analysis may be paused or delayed due to changes in hospital or university policies, federal, state or local regulations, prioritization of hospital resources toward pandemic efforts, or other reasons related to the pandemic. If the coronavirus continues to spread, some participants and clinical investigators may not be able to comply with clinical trial protocols. For example, quarantines or other travel limitations (whether voluntary or required) may impede participant movement, affect sponsor access to study

sites, or interrupt healthcare services, and we may be unable to conduct our clinical trials. Infections and deaths related to the pandemic may disrupt the United States' and other countries' healthcare and healthcare regulatory systems. Such disruptions could divert healthcare resources away from, or materially delay FDA or other regulatory review and/or approval with respect to, our clinical trials. It is unknown how long these disruptions could continue, were they to occur. Any elongation or de-prioritization of our clinical trials or delay in regulatory review resulting from such disruptions could materially affect the development and study of our product candidates.

We currently rely on third parties, such as contract laboratories, contract research organizations, medical institutions and clinical investigators to conduct these studies and clinical trials. If these third parties themselves are adversely impacted by restrictions resulting from the coronavirus outbreak, we will likely experience delays and/or realize additional costs. We also rely on third parties for the manufacture of our product candidates for preclinical and clinical testing. Disruptions to the global supply chain could impact our or our third-party manufacturers' ability to obtain raw materials or other products necessary to manufacture and distribute our product candidates. As a result, our efforts to obtain regulatory approvals for, and to commercialize, our product candidates may be delayed or disrupted.

The potential economic impact brought by, and the duration of, a resurgence of the pandemic may be difficult to assess or predict, however it has already caused, and is likely to result in further, significant disruption of global financial markets, which may reduce our ability to access capital either at all or on favorable terms. In addition, a recession, depression or other sustained adverse market event resulting from the continued spread of the coronavirus could materially and adversely affect our business and the value of our common stock.

The ultimate impact of the current pandemic, or any other health epidemic, is highly uncertain and will depend on future developments that cannot be predicted with confidence, such as the duration of the outbreak, the severity of COVID-19, and the effectiveness of actions to contain and treat for COVID-19. Although, as of the date of this Annual Report on Form 10-K, we do not expect any material impact on our long-term activity, we do not yet know the full extent of potential delays or impacts on our business, our clinical trials, our research programs, healthcare systems or the global economy as a whole, which could have a material adverse effect on our business, financial condition and results of operations and cash flows.

The ability of the Company's employees and consultants to work may be significantly impacted by a resurgence of the coronavirus.

Resurgence of the coronavirus can impact business recovery from the COVID-19 pandemic. This could require the Company to re-enact precautionary measures to help minimize the risk of our employees being exposed to a resurgence of the coronavirus and could compromise the ability of independent contractors who perform consulting services for us to deliver services or deliverables in a satisfactory or timely manner. Such a resurgence also would require our management team to focus on mitigating the adverse effects of the COVID-19 pandemic, which previously has required a large investment of time and resources, thereby diverting their attention from other priorities that existed prior to the outbreak of the pandemic. If these conditions worsen, or last for an extended period of time, the Company's ability to manage its business may be impaired, and operational risks, cybersecurity risks and other risks facing the Company even prior to the pandemic may be elevated.

Russian military action in Europe may impact foreign countries in which we may need to enroll patients in our clinical trials and could cause such clinical trials to be delayed or suspended.

In February 2022, Russia commenced a military invasion of Ukraine. Russia's invasion and the ensuing response by Ukraine may disrupt our ability to conduct clinical trials in Russia, Ukraine, and the neighboring areas. Although the route, length and impact of Russia's military action is highly unpredictable, clinical trial sites in Russia, Ukraine, Belarus, and Georgia may suspend or terminate trials, and patients could be forced to evacuate or choose to relocate, making them unavailable for initial or further participation in clinical trials. Alternative sites to fully and timely compensate for our clinical trial activities in these areas may not be available and we may need to find other countries to conduct these clinical trials. Furthermore, Russian military action may prevent the FDA from auditing clinical sites in these countries. Interruptions of clinical trials may delay our clinical development and approvals for our product candidates, which could increase our costs and jeopardize our ability to commence product sales and generate revenues.

Our growth is subject to economic and political conditions.

Our business is affected by global and local economic and political conditions as well as the state of the financial markets, inflation, recession, financial liquidity, currency volatility, growth, and policy initiatives. There can be no assurance that global economic conditions and financial markets will not worsen and that we will not experience any adverse effects that may be material to our consolidated cash flows, results of operations, financial position or our ability to access capital, such as the adverse effects resulting from a prolonged shutdown in government operations both in the United States and internationally. Political changes, including war or other conflicts, some of which may be disruptive, could interfere with our supply chain, our customers and all of our activities in a particular location.

We may not be able to manage our business effectively if we are unable to attract and retain key personnel.

We may not be able to attract or retain qualified management and commercial, scientific and clinical personnel in the future due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses. If we are not able to attract and retain necessary personnel to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development objectives, our ability to raise additional capital and our ability to implement our business strategy.

Our employees, consultants, or third-party partners may engage in misconduct or other improper activities, including but not necessarily limited to noncompliance with regulatory standards and requirements or internal procedures, policies or agreements to which such employees, consultants and partners are subject, any of which could have a material adverse effect on our business.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees, consultants, or third-party partners could include intentional failures to comply with FDA regulations, provide accurate information to the FDA, comply with cGMPs, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately, comply with internal procedures, policies or agreements to which such employees, consultants or partners are subject, 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, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee, consultant, or third-party misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation, as well as civil and criminal liability. The precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such 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 and results of operations, including the imposition of significant fines or other civil and/or criminal sanctions.

We receive a large amount of proprietary information from potential or existing licensors of intellectual property and potential acquisition target companies, all pursuant to confidentiality agreements. The confidentiality and proprietary invention assignment agreements that we have in place with each of our employees and consultants prohibit the unauthorized disclosure of such information, but such employees or consultants may nonetheless disclose such information through negligence or willful misconduct. Any such unauthorized disclosures could subject us to monetary damages and/or injunctive or equitable relief. The notes, analyses and memoranda that we have generated based on such information are also valuable to our businesses, and the unauthorized disclosure or misappropriation of such materials by our employees and consultants could significantly harm our strategic initiatives – especially if such disclosures are made to our competitors.

We rely on information technology, and any internet or internal computer system failures, inadequacies, interruptions or compromises of our systems or the security of confidential information could damage our reputation and harm our business.

Although a significant portion of our business is conducted using traditional methods of contact and communications such as face-to-face meetings, our business is increasingly dependent on critical, complex and interdependent information technology systems, including internet-based systems, to support business processes as well as internal and external communications. We could experience system failures and degradations in the future. We cannot assure you that we will be able to prevent an extended and/or material system failure if any of the following or similar events occurs:

- human error;
- subsystem, component, or software failure;
- a power or telecommunications failure;
- hacker attacks, cyber-attacks, software viruses, security breaches, unauthorized access or intentional acts of vandalism; or
- terrorist acts or war.

If any of the foregoing events were to occur, our business operations could be disrupted in ways that would require the incurrence of substantial expenditures to remedy. Any system failure, accident or security breach that causes interruptions in our operations could result in a material disruption of our drug development programs. For example, the loss of clinical trial data from completed clinical trials for one

or more of our product conducts could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data and applications, or inappropriate/unauthorized disclosure of confidential or proprietary information (including trade secrets), we could incur liability and our business and financial condition could be harmed.

The occurrence of a catastrophic disaster could damage our facilities beyond insurance limits, or we could lose key data which could cause us to curtail or cease operations.

We are vulnerable to damage and/or loss of vital data from natural disasters, such as earthquakes, tornadoes, power loss, fire, health epidemics and pandemics, floods and similar events, as well as from accidental loss or destruction. If any disaster were to occur, our ability to operate our businesses could be seriously impaired. We have property, liability and business interruption insurance that may not be adequate to cover losses resulting from disasters or other similar significant business interruptions, and we do not plan to purchase additional insurance to cover such losses due to the cost of obtaining such coverage. Any significant losses that are not recoverable under our insurance policies could seriously impair our business, financial condition and prospects. Any of the aforementioned circumstances, including without limitation the resurgence of COVID-19 virus, may also impede our employees' and consultants' abilities to provide services in-person and/or in a timely manner; hinder our ability to raise funds to finance our operations on favorable terms or at all; and trigger effectiveness of "force majeure" clauses under agreements with respect to which we receive goods and services, or under which we are obligated to achieve developmental milestones on certain timeframes. Disputes with third parties over the applicability of such "force majeure" clauses, or the enforceability of developmental milestones and related extension mechanisms in light of such business interruptions, may arise and may become expensive and time-consuming.

Our stock may be subject to substantial price and volume fluctuations due to a number of factors, many of which are beyond our control and may prevent our stockholders from reselling our common stock at a profit.

The market prices for securities of biotechnology and pharmaceutical companies have historically been highly volatile, and the market has from time to time experienced significant price and volume fluctuations that are unrelated to the operating performance of particular companies.

The market price of our common stock is likely to be highly volatile and may fluctuate substantially due to many factors, including:

- announcements concerning the progress of our efforts to obtain regulatory approval for and commercialize our product candidates or any future product candidate, including any requests we receive from the FDA for additional studies or data that result in delays in obtaining regulatory approval or launching these product candidates, if approved;
- market conditions in the pharmaceutical and biotechnology sectors or the economy as a whole;
- price and volume fluctuations in the overall stock market;
- the failure of one or more of our product candidates or any future product candidate, if approved, to achieve commercial success;
- announcements of the introduction of new products by us or our competitors;
- developments concerning product development results or intellectual property rights of others;
- litigation or public concern about the safety of our potential products;
- actual fluctuations in our quarterly operating results, and concerns by investors that such fluctuations may occur in the future;
- deviations in our operating results from the estimates of securities analysts or other analyst comments;
- additions or departures of key personnel;
- health care reform legislation, including measures directed at controlling the pricing of pharmaceutical products, and third-party coverage and reimbursement policies;

- developments concerning current or future strategic collaborations; and
- discussion of us or our stock price by the financial and scientific press and in online investor communities.

We may become involved in securities class action litigation that could divert management's attention and harm our business.

The stock markets have from time to time experienced significant price and volume fluctuations that have affected the market prices for the common stock of biotechnology and pharmaceutical companies. These broad market fluctuations may cause the market price of our stock to decline. In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biotechnology and biopharmaceutical companies have experienced significant stock price volatility in recent years. We may become involved in this type of litigation in the future. Litigation often is expensive and diverts management's attention and resources, which could adversely affect our business.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

Our corporate and executive office is located at 377 Plantation Street, Worcester, MA 01605.

On October 27, 2017, we entered into a lease agreement with WCS - 377 Plantation Street, Inc., a Massachusetts nonprofit corporation ("Landlord"). Pursuant to the terms of the lease agreement, we agreed to lease 27,043 sf from the Landlord, located at 377 Plantation Street in Worcester, MA (the "Facility"), through November 2026, subject to additional extensions at our option. Base rent, net of abatements of $0.6 million over the lease term, totals approximately $3.6 million, on a triple-net basis.

The Facility became operational for the production of personalized CAR T and gene therapies in 2018.

Item 3. Legal Proceedings

We are not involved in any litigation that we believe could have a material adverse effect on our financial position or results of operations. There is no action, suit, proceeding, inquiry or investigation before or by any court, public board, government agency, self-regulatory organization or body pending or, to the knowledge of our executive officers, threatened against or affecting our company or our officers or directors in their capacities as such.

Item 4. Mine Safety Disclosures

Not applicable

PART II

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

Market information

Our common stock has been quoted on the NASDAQ Global Market since August 22, 2017, under the symbol "MBIO." Prior to this there was no public market for our common stock.

Securities Authorized for Issuance Under Equity Compensation Plans

On November 30, 2017, we filed a registration statement on Form S-8 under the Securities Act registering the common stock issued, issuable or reserved for issuance under our 2016 Plan. That registration statement became effective immediately upon filing, and shares covered by the registration statement are eligible for sale in the public markets, subject to grant of the underlying awards, vesting provisions and Rule 144 limitations applicable to our affiliates.

The information required by Item 5 of Form 10-K addressing equity compensation plans is incorporated herein by reference to "Item 12, Security Ownership of Certain Beneficial Owners of Management and Related Stockholder Matters."

Holders of Record

As of December 31, 2021, there were approximately 86 holders of record of our common stock and one holder of record for our Class A common stock. The actual number of stockholders of our common shares is greater than this number of record holders and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividends

We have never paid or declared any cash dividends on our common stock, and we do not anticipate paying any cash dividends on our common stock in the foreseeable future. We intend to retain all available funds and any future earnings to fund the development and expansion of our business. Any future determination to pay dividends will be at the discretion of our board of directors and will depend upon a number of factors, including our results of operations, financial condition, future prospects, contractual restrictions, restrictions imposed by applicable law and other factors our board of directors deems relevant.

Recent Sales of Unregistered Securities

None.

Item 6. Reserved

Item 7. Management's Discussion and Analysis of the Results of Operations

Statements in the following discussion and throughout this report that are not historical in nature are "forward-looking statements." You can identify forward-looking statements by the use of words such as "expect," "anticipate," "estimate," "may," "will," "should," "intend," "believe," and similar expressions. Although we believe the expectations reflected in these forward-looking statements are reasonable, such statements are inherently subject to risk and we can give no assurances that our expectations will prove to be correct. Actual results could differ from those described in this report because of numerous factors, many of which are beyond our control. These factors include, without limitation, those described under Item 1A "Risk Factors." We undertake no obligation to update these forward-looking statements to reflect events or circumstances after the date of this report or to reflect actual outcomes. Please see "Forward-Looking Statements" at the beginning of this Form 10-K.

The following discussion of our financial condition and results of operations should be read in conjunction with our financial statements and the related notes thereto and other financial information appearing elsewhere in this Form 10-K. We undertake no obligation to update any forward-looking statements in the discussion of our financial condition and results of operations to reflect events or circumstances after the date of this report or to reflect actual outcomes.

Overview

Mustang Bio, Inc. ("Mustang," "We," "Us" or the "Company") is a clinical-stage biopharmaceutical company focused on translating today's medical breakthroughs in cell and gene therapies into potential cures for hematologic cancers, solid tumors and rare genetic diseases. We aim to acquire rights to these technologies by licensing or otherwise acquiring an ownership interest in the technologies, funding their research and development and eventually either out-licensing or bringing the technologies to market.

Our pipeline is currently focused in three core areas: gene therapies for rare genetic disorders, chimeric antigen receptor ("CAR") engineered T cell ("CAR T") therapies for hematologic malignancies and CAR T therapies for solid tumors. For each therapy we have partnered with world class research institutions. For our gene therapies, we have partnered with St. Jude Children's Research Hospital ("St. Jude") in the development of a first-in-class *ex vivo* lentiviral treatment of X-linked severe combined immunodeficiency ("XSCID") and with Leiden University Medical Centre ("LUMC") for RAG1 severe combined immunodeficiency ("RAG1-SCID"). For our CAR T therapies we have partnered with the City of Hope National Medical Center ("COH"), Fred Hutchinson Cancer Research Center ("Fred Hutch"), Nationwide Children's Hospital ("Nationwide") and the Mayo Foundation for Medical Education and Research ("Mayo Clinic").

Gene Therapies

In partnership with St. Jude, our XSCID gene therapy programs (MB-107 and MB-207) are being conducted under an exclusive license to develop a potentially curative treatment for XSCID, a rare genetic immune system condition in which affected patients do not live beyond infancy without treatment. This first-in-class *ex vivo* lentiviral gene therapy is currently in two Phase 1/2 clinical trials involving two different autologous cell products: a multicenter trial of the MB-107 product in newly diagnosed infants sponsored by St. Jude and a single-center trial of the MB-207 product in previously transplanted patients sponsored by the National Institutes of Health ("NIH"). In January 2021 we received approval to proceed with our Investigational New Drug ("IND") application with the U.S. Food and Drug Administration ("FDA") to initiate a pivotal non-randomized multicenter Phase 2 clinical trial of MB-107 in newly diagnosed infants with XSCID who are under the age of two. We expect to enroll the first patient in a pivotal multicenter Phase 2 clinical trial in the second half of 2022. In January 2022, the FDA issued a hold, pending Chemistry, Manufacturing and Controls ("CMC") clearance, on our IND application to conduct a pivotal non-randomized multicenter Phase 2 clinical trial of MB-207 in previously transplanted XSCID patients. In order to lift this hold and receive FDA clearance for the IND, we believe the most critical activities will be to (1) perform process validation manufacturing runs using healthy donor material and (2) ensure qualification of all assays related to the product release. We estimate that these activities will take 3-6 months to complete, and we therefore expect to enroll the first patient in a pivotal multicenter Phase 2 clinical trial in the first quarter of 2023.

CAR T Therapies

Our pipeline of CAR T therapies is being developed under exclusive licenses from several world class research institutions. Our strategy is to license these technologies, support preclinical and clinical research activities by our partners and transfer the underlying technology to our cell processing facility located in Worcester, Massachusetts, in order to conduct our own clinical trials.

We are developing CAR T therapies for hematologic malignancies in partnership with COH targeting CD123 (MB-102) and CS1 (MB-104) and with Fred Hutch targeting CD20 (MB-106). Phase 1 clinical trials sponsored by COH for MB-102 and MB-104 and by Fred Hutch for MB-106 are underway. In the third quarter of 2019 the FDA approved our IND application to initiate a multi-center Phase 1/2 clinical trial of MB-102, and our clinical trial began enrollment in 2020 for the treatment of patients with blastic plasmacytoid dendritic cell neoplasm. In May 2021, the FDA approved our IND application to initiate a multi-center Phase 1/2 clinical trial of MB-106, and we expect to begin the treatment of patients with non-Hodgkin lymphoma and chronic lymphocytic leukemia in the first half of 2022. We plan to file an IND for a multicenter Phase 1/2 trial for MB-104 for the treatment of patients with multiple myeloma once COH has established a safe and effective dose.

We are also developing CAR T therapies for solid tumors in partnership with COH targeting IL13Rα2 (MB-101), HER2 (MB-103) and PSCA (MB-105). In addition, we have partnered with Nationwide for the C134 oncolytic virus (MB-108) in order to enhance the activity of MB-101 for the treatment of patients with glioblastoma multiforme ("GBM"). Phase 1 clinical trials sponsored by COH for MB-101, MB-103 and MB-105 are underway. A Phase 1 clinical trial sponsored by the University of Alabama at Birmingham ("UAB") for MB-108 began during the third quarter of 2019 and, in the second half of 2022, we plan to file an IND for the combination of MB-101 and MB-108 – which is referred to as MB-109 – for the treatment of patients with relapsed or refractory GBM and anaplastic astrocytoma. We also plan to file INDs and initiate our own clinical trials for MB-103 for the treatment of patients with metastatic breast cancer to brain and for MB-105 for the treatment of patients with prostate and pancreatic cancer. The Company is also collaborating with the Mayo Clinic to develop a novel technology that may be able to transform the administration of CAR T therapies and potentially be used as an off-the-shelf therapy. Mustang plans to file an IND application for a multicenter Phase 1 clinical trial once a lead construct has been identified.

Recent Events

Leiden University Medical Centre License

On November 10, 2021, the Company announced that it has executed an exclusive license agreement with Leiden University Medical Centre ("LUMC") for a first-in-class ex vivo lentiviral gene therapy for the treatment of RAG1 severe combined immunodeficiency ("RAG1-SCID"). The therapy, which includes low-dose conditioning prior to reinfusion of the patients' own gene-modified blood stem cells, is currently being evaluated in a Phase 1/2 multicenter clinical trial in Europe. The ongoing clinical trial recently enrolled its first patient, and additional clinical sites are expected to be added in the near future. The RAG1-SCID program has been granted Orphan Drug Designation by the European Medicines Agency.

The Company also established an ongoing partnership with Frank J. Staal, Ph.D., professor of Molecular Stem Cell Biology and molecular immunologist at LUMC, whose laboratory developed the therapy. Dr. Staal will continue the development of additional lentiviral gene therapies in his lab, to which we have rights under the agreement.

The RAG1-SCID therapy expands the pipeline of ex vivo lentiviral gene therapies we are currently developing. The lead programs, MB-107 and MB-207, are being investigated for the treatment of XSCID. A pivotal multicenter trial studying MB-107 is expected to enroll its first patient in the first quarter of 2022. Combined, XSCID and RAG1-SCID make up almost 60% of all SCID cases combined.

MB-106 (CD20-targeted CAR T for Non-Hodgkin Lymphoma and Chronic Lymphocytic Leukemia)

On November 4, 2021, the Company announced that interim Phase 1/2 data on MB-106 have been selected for a poster presentation at the 63rd American Society of Hematology Annual Meeting ("ASH2021"), which was held from December 11-14, 2021.

The abstract posted on the ASH2021 website reported on 16 patients [12 follicular lymphoma (FL), 2 mantle cell lymphoma (MCL), 1 CLL and 1 diffuse large B-cell lymphoma (DLBCL)] treated following a major cell manufacturing process modification. CAR T cells are administered at one of 4 dose levels (DL): DL1: 3.3x105, DL2: 1x106, DL3: 3.3x106, DL4: 1x107 CAR T cells/kg. All DLs were reached, with no dose-limiting toxicities observed to date.

The overall response rate (ORR) was 94% (15/16) with a complete response (CR) rate of 62% (10/16). In patients with FL (n=12), ORR was 92% (11/12) and CR rate was 75% (9/12). Among patients with FL who received DL 3 or 4, the CR rate was 86%. The patient with CLL had a PET-negative CR and undetectable measurable residual disease in peripheral blood and bone marrow by flow cytometry at a sensitivity of 10-4 (uMRD4) on day 28. The patient with DLBCL achieved a partial response (PR) on day 28, and a repeat PET on day ~90 showed deepening of the PR. Among patients who achieved a CR, only one patient with FL relapsed after 9 months. All other CRs are ongoing (range: 3-18 months). CAR T persistence was lost at day 95 in one patient who had progression and proceeded to other anti-lymphoma treatment; 2 other patients lost CAR T engraftment by day 181 and 201 with B-cell recovery. All other patients continue to have detectable CAR T cells as of last follow-up (maximum of 13 months post-infusion).

Among the 16 total patients reported in the abstract, there were seven occurrences of cytokine release syndrome (4 patients with Grade 1 and 3 patients with Grade 2) and one occurrence of Grade 2 immune effector cell-associated neurotoxicity syndrome. One patient with CLL developed Grade 3 temporary neuropathic pain which, in the absence of other explanation, was attributed to CAR T therapy. No patients had tumor lysis syndrome or Grade 3-4 infections. Thrombocytopenia (Grade 3-4: 19%) and neutropenia (Grade 3-4: 94%) were common, but there were no bleeding complications, and the rate of febrile neutropenia was 19%.

NIH Grant For MB-106 CD20-Targeted CAR T Cell Therapy

On November 1, 2021, we announced that the Company was awarded a grant of approximately $2 million from the National Cancer Institute. This two-year grant will partially fund the Phase 1, Open Label, Multicenter Trial to Assess the Safety, Tolerability and Efficacy of MB-106, a CD20-targeted, autologous CAR T cell therapy for patients with relapsed or refractory NHL or CLL.

Mayo Clinic License

In August 2021, we announced an exclusive license agreement with the Mayo Foundation for Medical Education and Research (the "Mayo Clinic") for a novel technology that may be able to transform the administration of CAR T therapies and has the potential to be used as an off-the-shelf therapy.

The technology, developed by Larry R. Pease, Ph.D., principal investigator and former director of the Center for Immunology and Immune Therapies at Mayo Clinic, is a new platform to administer CAR T therapy using a two-step approach. First, a peptide is administered to the patient to drive the proliferation of the patient's resident T cells. This is followed by the administration of a viral CAR construct directly into the lymph nodes of the patient. In turn, the viral construct infects the activated T cells and effectively forms CAR T cells *in vivo* in the patient. Successful implementation may lead to an off-the-shelf product with no need to isolate and expand patient T cells *ex vivo*.

Preclinical proof-of-concept has been established, and the ongoing development of this technology will take place at Mayo Clinic. Mustang plans to file an Investigational New Drug ("IND") application for a multicenter Phase 1 clinical trial once a lead construct has been identified.

MB-107 and MB-207 (Ex vivo Lentiviral Therapy for X-linked Severe Combined Immunodeficiency (XSCID))

In August 2021, we announced that the European Medicines Agency ("EMA") granted Priority Medicines ("PRIME") designation to MB-107, a lentiviral gene therapy for the treatment of XSCID in newly diagnosed infants. In addition to PRIME designation, the EMA granted Advanced Therapy Medicinal Product ("ATMP") classification to MB-107 in April 2020 and Orphan Drug designation in November 2020.

MB-107 has also received Orphan Drug, Rare Pediatric Disease and Regenerative Medicine Advanced Therapy ("RMAT") designations from the U.S. Food and Drug Administration ("FDA").

The FDA grants Rare Pediatric Disease Designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and affect fewer than 200,000 people in the United States. If Mustang's BLA for MB-107 is approved, the Company may be eligible to receive a priority review voucher, which can be redeemed to obtain priority review for any subsequent marketing application and may be sold or transferred. This program is intended to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases.

The FDA has also granted Rare Pediatric Disease Designation for MB-207. If Mustang's BLA for MB-207 is approved, the Company may be eligible to receive a priority review voucher for this product as well, which can also be redeemed to obtain priority review for any subsequent marketing application and may be sold or transferred. MB-207 has also received Orphan Drug designation from the FDA and has received Orphan Drug designation and ATMP classification from the EMA.

Registration Statements

On April 23, 2021, the Company filed a shelf registration statement No. 333-255476 on Form S-3 (the "2021 S-3"), which was declared effective on May 24, 2021. Under the 2021 S-3, the Company may sell up to a total of $200.0 million of its securities. As of December 31, 2021, there have been no sales of securities under the 2021 S-3.

On October 23, 2020, the Company filed a shelf registration statement No. 333-249657 on Form S-3 (the "2020 S-3"), which was declared effective on December 4, 2020. Under the 2020 S-3, the Company may sell up to a total of $100.0 million of its securities. As of December 31, 2021, approximately $14.6 million of the 2020 S-3 remains available for sales of securities.

Term Loan

On March 29, 2019 (the "Closing Date"), the Company entered into a $20.0 million Loan Agreement with Horizon (the "Loan Agreement"), the proceeds of which were used to provide the Company with additional working capital to continue development of its gene and cell therapies. In accordance with the Loan Agreement, $15.0 million of the $20.0 million loan was funded on the Closing Date, with the remaining $5.0 million fundable upon the Company achieving certain predetermined milestones. On September 30, 2020, the Company repaid in full all amounts that were outstanding under the Loan Agreement.

At-the-Market Offering

On July 13, 2018, the Company filed a shelf registration statement No. 333-226175 on Form S-3, as amended on July 20, 2018 (the "2018 S-3"), which was declared effective in August 2018. Under the 2018 S-3, the Company may sell up to a total of $75.0 million of its securities. In connection with the 2018 S-3, the Company entered into an At-the-Market Issuance Sales Agreement (the "ATM Agreement") with B. Riley Securities, Inc. (formerly B. Riley FBR, Inc.), Cantor Fitzgerald & Co., National Securities Corporation, and Oppenheimer & Co. Inc. (each an "Agent" and collectively, the "Agents"), relating to the sale of shares of common stock. Under the ATM Agreement, the Company pays the Agents a commission rate of up to 3.0% of the gross proceeds from the sale of any shares of common stock. On December 31, 2020, the ATM Agreement was amended to add H.C. Wainwright & Co., LLC as an Agent.

During the year ended December 31, 2021, the Company issued approximately 19.4 million shares of common stock at an average price of $3.70 per share for gross proceeds of $71.9 million under the ATM Agreement. In connection with these sales, we paid aggregate fees of approximately $1.3 million for net proceeds of approximately $70.6 million.

During the year ended December 31, 2020, the Company issued approximately 17.6 million shares of common stock at an average price of $3.40 per share for gross proceeds of $59.8 million under the ATM Agreement. In connection with these sales, we paid aggregate fees of approximately $1.1 million for net proceeds of approximately $58.7 million.

Authorized Shares

On June 17, 2021, the stockholders of the Company voted at the 2021 Annual Meeting to approve an amendment to Mustang's Amended and Restated Certificate of Incorporation to increase the number of shares of common stock authorized for issuance by 25 million shares, bringing the total number of authorized shares of common stock to 150 million shares.

To date, we have not received approval for the sale of our product candidates in any market and, therefore, have not generated any product sales from our product candidates. In addition, we have incurred substantial operating losses since our inception, and expect to continue to incur significant operating losses for the foreseeable future and may never become profitable. As of December 31, 2021, we have an accumulated deficit of $251.8 million.

We are a majority-controlled subsidiary of Fortress Biotech, Inc. ("Fortress"). As a "Controlled Company" we rely on the exemption provided by Nasdaq Listing Rule 5615(c)(2), which permits us to maintain less than a majority of independent directors on our board.

Critical Accounting Policies and Use of Estimates

The Company's financial statements include certain amounts that are based on management's best estimates and judgments. The Company's significant estimates include, but are not limited to, useful lives assigned to long-lived assets and amortizable intangible assets, fair value of stock options and warrants, stock-based compensation, accrued expenses, provisions for income taxes and contingencies. Due to the uncertainty inherent in such estimates, actual results may differ from these estimates. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources.

Actual results may differ from these estimates under different assumptions or conditions. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

While our significant accounting policies are described in the notes to our financial statements included elsewhere in this Report, we believe that the following critical accounting policies are most important to understanding and evaluating our reported financial results.

Research and Development

Research and development costs are expensed as incurred. Advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made. Upfront and milestone payments due to third parties that perform research and development services on the Company's behalf will be expensed as services are rendered or when the milestone is achieved.

Research and development costs primarily consist of personnel related expenses, including salaries, benefits, travel, and other related expenses, stock-based compensation, payments made to third parties for license and milestone costs related to in-licensed products and technology, payments made to third party contract research organizations for preclinical and clinical studies, investigative sites for clinical trials, consultants, the cost of acquiring and manufacturing clinical trial materials, and costs associated with regulatory filings, laboratory costs and other supplies.

In accordance with ASC 730 10 25 1, *Research and Development*, costs incurred in obtaining technology licenses are charged to research and development expense if the technology licensed has not reached commercial feasibility and has no alternative future use. In each case, we evaluate if the license agreement results in the acquisition of an asset or a business. Such licenses purchased by the Company require substantial completion of research and development, regulatory and marketing approval efforts in order to reach commercial feasibility and has no alternative future use. Accordingly, the total purchase price for the licenses acquired during the period was reflected as research and development - licenses acquired on the Statements of Operations for the years ended December 31, 2021 and 2020.

Accrued Research and Development Expense

We record accruals for estimated costs of research, preclinical, clinical and manufacturing development within accrued expenses which are significant components of research and development expenses. A substantial portion of our ongoing research and development activities is conducted by third-party service providers. We accrue the costs incurred under agreements with these third parties based on estimates of actual work completed in accordance with the respective agreements. We determine the estimated costs through discussions with internal personnel and external service providers as to the progress, or stage of completion or actual timeline (start-date and end-date) of the services and the agreed-upon fees to be paid for such services. Payments made to third parties under these arrangements in advance of the performance of the related services are recorded as prepaid expenses until the services are rendered.

If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust accrued expenses or prepaid expenses accordingly, which impact research and development expenses. Although we do not expect our estimates to be materially different

from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period.

Fair Value Measurement

The Company follows accounting guidance on fair value measurements for financial assets and liabilities measured at fair value on a recurring basis. Under the accounting guidance, fair value is defined as an exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or a liability.

The accounting guidance requires fair value measurements be classified and disclosed in one of the following three categories:

Level 1: Quoted prices in active markets for identical assets or liabilities.
Level 2: Observable inputs other than Level 1 prices for similar assets or liabilities that are directly or indirectly observable in the marketplace.
Level 3: Unobservable inputs which are supported by little or no market activity and that are financial instruments whose values are determined using pricing models, discounted cash flow methodologies, or similar techniques, as well as instruments for which the determination of fair value requires significant judgment or estimation.

The fair value hierarchy also requires an entity to maximize the use of observable inputs and minimize the use of unobservable inputs when measuring fair value. Assets and liabilities measured at fair value are classified in their entirety based on the lowest level of input that is significant to the fair value measurement. The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires management to make judgments and consider factors specific to the asset or liability.

Certain of the Company's financial instruments are not measured at fair value on a recurring basis but are recorded at amounts that approximate their fair value due to their liquid or short-term nature, such as accounts payable, accrued expenses and other current liabilities.

Stock-Based Compensation

The Company expenses stock-based compensation to employees and non-employees over the requisite service period based on the estimated grant-date fair value of the awards and forfeitures, which are recorded upon occurrence. The Company estimates the fair value of stock option grants using the Black-Scholes option pricing model. The assumptions used in calculating the fair value of stock-based awards represent management's best estimates and involve inherent uncertainties and the application of management's judgment.

We will continue to use judgment in evaluating the expected volatility, expected terms and interest rates utilized for our stock-based compensation expense calculations on a prospective basis. The assumptions underlying these valuations represent our management's best estimate, which involve inherent uncertainties and the application of management judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, our stock-based compensation expense could be materially different. We expect to continue to grant options and other stock-based awards in the future, and to the extent that we do, our stock-based compensation expense recognized in future periods will likely increase.

Income Taxes

The Company accounts for income taxes under ASC 740, *Income Taxes* ("ASC 740"). ASC 740 requires the recognition of deferred tax assets and liabilities for both the expected impact of differences between the financial statement and tax basis of assets and liabilities and for the expected future tax benefit to be derived from tax loss and tax credit carry forwards. ASC 740 additionally requires a valuation allowance to be established when it is more likely than not that all or a portion of deferred tax assets will not be realized. Judgments concerning the recognition and measurement of a tax benefit might change as new information becomes available. Our unrecognized tax benefits, if recognized, would not have an impact on our effective tax rate assuming we continue to maintain a full valuation allowance position. We do not expect our unrecognized tax benefits to change significantly over the next 12 months.

ASC 740 also clarifies the accounting for uncertainty in income taxes recognized in an enterprise's financial statements and prescribes a recognition threshold and measurement process for financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. For those benefits to be recognized, a tax position must be more-likely-than-not to be sustained upon examination by taxing authorities. ASC 740 also provides guidance on de-recognition, classification, interest and penalties, accounting in interim period, disclosure and transition. Based on the Company's evaluation, it has been concluded that there are no significant uncertain tax positions

requiring recognition in the Company's financial statements. The 2018 through 2020 tax years are the only periods subject to examination upon filing of appropriate tax returns. The Company believes that its income tax positions and deductions would be sustained on audit and does not anticipate any adjustments that would result in a material change to its financial position.

The Company's policy for recording interest and penalties associated with audits is to record such expense as a component of income tax expense. There were no amounts accrued for penalties or interest as of or during the years ended December 31, 2021 and 2020. Management is currently unaware of any issues under review that could result in significant payments, accruals or material deviations from its position.

Recent Accounting Pronouncements

See Note 2 to the Financial Statements.

Smaller Reporting Company Status

We are a "smaller reporting company," meaning that the market value of our shares held by non-affiliates is less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company if either (i) the market value of our shares held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than $700 million. 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 , have reduced disclosure obligations regarding executive compensation, and smaller reporting companies are permitted to delay adoption of certain recent accounting pronouncements discussed in Note 2 to our consolidated financial statements located in "Part IV, Item 15., Exhibits and Financial Statement Schedules" in this Annual Report on Form 10-K.

Results of Operations

Comparison of the Years Ended December 31, 2021 and 2020

	For the year ended December 31,		Change	
($in thousands)	**2021**	**2020**	**$**	**%**
Operating expenses:				
Research and development	$ 49,864	$ 37,237	$ 12,627	34 %
Research and development – licenses acquired	5,842	10,064	(4,222)	(42)%
General and administrative	11,017	9,505	1,512	16 %
Total operating expenses	66,723	56,806	9,917	17 %
Loss from operations	(66,723)	(56,806)	(9,917)	17 %
Other income (expense)				
Interest income	368	708	(340)	(48)%
Interest expense	(15)	(3,917)	3,902	(100)%
Total other income (expense)	353	(3,209)	3,562	(111)%
Net Loss	**$ (66,370)**	**$ (60,015)**	**$ (6,355)**	**11 %**

Research and Development Expenses

Research and development expenses primarily consist of personnel related expenses, including salaries, benefits, travel, and other related expenses, stock-based compensation, payments made to third parties for license, sponsored research and milestone costs related to in-licensed products and technology, payments made to third party contract research organizations for preclinical and clinical studies, investigative sites for clinical trials, consultants, the cost of acquiring and manufacturing clinical trial materials, costs associated with regulatory filings, laboratory costs and other supplies.

Research and development expense increased by approximately $12.6 million from $37.2 million for the year ended December 31, 2020 to $49.8 million for the year ended December 31, 2021. The increase in research and development expense for the year ended December 31, 2021 was primarily attributable to the following:

- $4.5 million for increased research and development employee compensation costs, including stock compensation, as we continue to increase research and development headcount to support development of our clinical programs;

- $3.9 million for increased laboratory supply costs;
- $2.3 million for increased consulting costs primarily to support Mustang-sponsored clinical trials;
- approximately $3.0 million for increased other costs including third-party contract research organizations, outside services, vector manufacturing and depreciation; and
- offset by approximately $1.1 million for decreased costs for sponsored research and clinical trial agreements.

Research and development expenses - licenses acquired decreased by $4.2 million from $10.1 million for the year ended December 31, 2020 to $5.9 million for the year ended December 31, 2021. The decrease in research and development expenses - licenses acquired for the year ended December 31, 2021 was primarily attributable to the following:

- Approximately $3.4 million for the annual stock dividend to Fortress;
- $1.4 million related to our licenses with COH;
- $0.3 million related to our CD20 license with Fred Hutch;
- $0.1 million related to our CSL Behring (Calimmune);
- $0.1 million related to our LentiBOOST™ license with SIRION; and
- offset by approximately $1.1 million for increased costs related to our licenses with Mayo Clinic and Leiden University Medical Centre.

We expect our research and development activities to increase as we develop our existing product candidates and potentially acquire new product candidates, reflecting increasing costs associated with the following:

- employee-related expenses, which include salaries and benefits;
- license fees and milestone payments related to in-licensed products and technology;
- expenses incurred under agreements with contract research organizations, investigative sites and consultants that conduct our clinical trials and our preclinical activities;
- facility expenses, which include rent, utilities and maintenance costs;
- the cost of acquiring and manufacturing clinical trial materials; and
- costs associated with non-clinical activities, and regulatory approvals.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related expenses, including stock-based compensation, for executives and other administrative personnel, recruitment expenses, professional fees and other corporate expenses, including investor relations, legal activities including patent fees, and facilities-related expenses.

General and administrative expense increased by approximately $1.5 million from $9.5 million for the year ended December 31, 2020 to $11.0 million for the year ended December 31, 2021. The increase in general and administrative expense for the year ended December 31, 2021 was primarily attributable to the following:

- $0.6 million for increased general and administrative employee compensation costs due primarily to additional headcount to support the Company’s continued growth;

- $0.8 million for increased state taxes;
- $0.3 million for increased audit and accounting fees;
- $0.9 million for increased other costs, including consulting and professional fees, outside services and insurance; and
- offset by approximately $1.1 million for decreased stock-based costs.

We anticipate general and administrative expenses will increase in future periods, reflecting continued and increasing costs associated with:

- support of our expanded research and development activities, including additional product candidates entering the clinic;
- stock compensation granted to key employees and non-employees;
- support of business development activities; and
- increased professional fees and other costs associated with the regulatory requirements and increased compliance associated with being a publicly traded company.

Other Income (Expense)

Other income (expense) consists primarily of interest income earned on cash balances and short-term investments and interest expense on the Company's notes payable. For the year ended December 31, 2021 and 2020, total other income (expense) were approximately $0.4 million of income and $3.2 million of expense, respectively. The $3.6 million increase in other income (expense) for the year ended December 31, 2021 was primarily attributable to lower interest expense of $3.9 million partially offset by lower interest income of $0.3 million.

Liquidity and Capital Resources

The Company has incurred substantial operating losses and expects to continue to incur significant operating losses for the foreseeable future and may never become profitable. As of December 31, 2021, the Company had an accumulated deficit of $251.8 million.

The Company has funded its operations to date primarily through the sale of equity, its Loan Agreement with Runway and its venture debt financing agreement (the "Horizon Loan Agreement") with Horizon Technology Finance Corporation ("Horizon"), herein referred to as the "Horizon Notes." In September 2020, we repaid the Horizon Notes in full all amounts that were outstanding under the Horizon Loan Agreement, which was comprised of $15.0 million face value of the outstanding notes, $112,500 accrued and unpaid interest, a $750,000 loan termination fee and prepayment penalties of $550,000. The Company expects to continue to use the proceeds from previous financing transactions primarily for general corporate purposes, including financing the Company's growth, developing new or existing product candidates, and funding capital expenditures, acquisitions and investments. The Company currently anticipates that its cash and cash equivalents balances at December 31, 2021, are sufficient to fund its anticipated operating cash requirements for at least one year from the date of this Form 10-K.

From January 1, 2022 through March 18, 2022, the Company issued approximately 2.4 million shares of common stock at an average price of $0.99 per share for gross proceeds of $2.4 million under the ATM Agreement.

The Company will be required to expend significant funds in order to advance the development of its product candidates. The Company will require additional financings through equity and debt offerings, collaborations and licensing arrangements or other sources to fully develop, prepare regulatory filings, obtain regulatory approvals and commercialize its existing and any new product candidates. If the Company is unable to arrange for such financings, or unable to arrange for them on terms acceptable to the Company, the Company's current development plans and plans for expansion of its facility and general and administrative infrastructure will be curtailed.

Cash Flows for the Years Ended December 31, 2021 and 2020

($in thousands)	For the year ended December 31, 2021	2020
Statement of cash flows data:		
Total cash (used in) provided by:		
Operating activities	$ (53,667)	$ (37,319)
Investing activities	(5,366)	(4,412)
Financing activities	70,847	78,122
Net change in cash, cash equivalents and restricted cash	$ 11,814	$ 36,391

Operating Activities

Net cash used in operating activities was $53.7 million for the year ended December 31, 2021, compared to $37.3 million for the year ended December 31, 2020. Net cash used in operating activities for the year ended December 31, 2021, was primarily due to approximately $66.4 million in net loss, partially offset by $4.2 million of common shares issuable for Founders shares, $3.3 million of non-cash stock compensation expenses, $2.2 million of depreciation expense, $1.9 million of equity fee on issuance of common shares to Fortress and $1.6 million of research and development-licenses acquired.

Net cash used in operating activities for the year ended December 31, 2020, was primarily due to approximately $60.0 million in net loss, partially offset by $7.6 million of common shares issuable for Founders shares, $3.1 million change in operating assets and liabilities, $3.0 million of non-cash stock compensation expenses, $2.5 million of research and development-licenses acquired, $2.4 million of equity fee on issuance of common shares to Fortress, $2.3 million of accretion of debt discount and $1.7 million of depreciation expense.

Investing Activities

Net cash used in investing activities was $5.4 million for the year ended December 31, 2021, representing $4.0 million in purchases of fixed assets and $1.4 million in purchases of research and development licenses.

Net cash provided by investing activities was $4.4 million for the year ended December 31, 2020, representing $2.5 million in purchases of research and development licenses and $1.9 million in purchases of fixed assets.

Financing Activities

Net cash provided by financing activities was $70.8 million during the year ended December 31, 2021, representing gross proceeds of $71.9 million, net of offering costs of $1.4 million, from the Mustang ATM and $0.3 million raised from the issuance of the Company's common shares in connection with the ESPP.

Net cash provided by financing activities was $78.1 million during the year ended December 31, 2020, representing gross proceeds of $59.8 million, net of offering costs of $1.1 million, from the Mustang ATM; gross proceeds of $37.2 million, net of offering costs of $2.4 million, from our June 2020 underwritten public offering and $0.3 million raised from the issuance of the Company's common shares in connection with the ESPP, partially offset by the prepayment of the Horizon Notes of $15.7 million.

Item 7A. Quantitative and Qualitative Disclosures About Market Risks

Not applicable.

Item 8. Financial Statements and Supplementary Data.

The information required by this Item is set forth in the financial statements and notes thereto beginning at page F-1 of this Annual Report on Form 10-K.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

Not applicable.

Item 9A. Controls and Procedures.

Disclosure Controls and Procedures

Controls and Procedures

Disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) are designed only to provide reasonable assurance that they will meet their objectives. Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness, as of December 31, 2021, of the design and operation of our disclosure controls and procedures, as such term is defined in Exchange Act Rules 13a-15(e) and 15d-15(e). Based on this evaluation, our principal executive officer and principal financial officer have concluded that, as of such date, our disclosure controls and procedures are effective to ensure that information required to be disclosed by us in our Exchange Act reports is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure.

Internal Control over Financial Reporting

Management's Report on Internal Control over Financial Reporting.

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rules 13a-15(f) and 15d-15(f). Internal control over financial reporting refers to the process designed by, or under the supervision of, our principal executive officer and principal financial officer, and effected by our Board of Directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles, and includes those policies and procedures that:

(1) pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets;

(2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles ("GAAP"), and that our receipts and expenditures are being made only in accordance with authorization of our management and directors; and

(3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisitions, use or disposition of our assets that could have a material effect on the financial statements.

Internal control over financial reporting has inherent limitations. Internal control over financial reporting is a process that involves human diligence and compliance and is subject to lapses in judgment and breakdowns resulting from human failures. Internal control over financial reporting also can be circumvented by collusion or improper management override. Because of such limitations, there is a risk that material misstatements may not be prevented or detected on a timely basis by internal control over financial reporting. However, these inherent limitations are known features of the financial reporting process. Therefore, it is possible to design into the process safeguards to reduce, though not eliminate, this risk.

Our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2021. In making the assessment, management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission ("COSO") in *Internal Control - Integrated Framework (2013)*.

Based on our assessment, our management has concluded that, as of December 31, 2021, our internal controls over financial reporting were effective based upon those criteria.

Changes in Internal Controls over Financial Reporting.

There were no changes in our internal control over financial reporting during our most recent fiscal quarter that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

None.

Item 9C. Disclosure Regarding Foreign Jurisdiction that Prevents Inspections.

None.

PART III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2022 Annual Meeting of Stockholders.

Item 11. Executive Compensation

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2022 Annual Meeting of Stockholders.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2022 Annual Meeting of Stockholders.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2022 Annual Meeting of Stockholders.

Item 14. Principal Accounting Fees and Services.

The information required by this Item is incorporated herein by reference from our Proxy Statement for our 2022 Annual Meeting of Stockholders.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

(a) Financial Statements.

The following financial statements are filed as part of this Form 10-K:

(b) Exhibits.

Exhibit No.	Description
3.1	Amended and Restated Certificate of Incorporation of Mustang Bio, Inc. (formerly Mustang Therapeutics, Inc.), dated July 26, 2016. * Filed as Exhibit 3.1 on the Company's Form 1012G filed on July 28, 2016.
3.2	Certificate of Amendment of the Amended and Restated Certificate of Incorporation of Mustang Bio, Inc., dated June 14, 2018. * Filed as Exhibit 3.1 on the Company's Form 10-Q filed on August 13, 2018.
3.3	Certificate of Amendment of the Amended and Restated Certificate of Incorporation of Mustang Bio, Inc., dated September 30, 2019. * Filed as Exhibit 3.1 on the Company's Form 8-K filed on September 30, 2019.
3.4	Certificate of Amendment of the Amended and Restated Certificate of Incorporation of Mustang Bio, Inc., dated December 4, 2020. * Filed as Exhibit 3.1 on the Company's Form 8K filed on December 4, 2020.
3.5	Certificate of Amendment of the Amended and Restated Certificate of Incorporation of Mustang Bio, Inc., dated June 17, 2021. * Filed as Exhibit 3.1 on the Company's Form 8-K filed on June 22, 2021.
3.6	Bylaws of Mustang Bio, Inc. * Filed as Exhibit 3.2 on the Company's Form 1012G filed on July 28, 2016.
4.1	Specimen certificates evidencing shares of common stock, Class A common stock and Class A preferred stock. * Filed as Exhibit 4.1 on the Company's Form 1012G filed on July 28, 2016.
4.2	Form of warrant agreement.* Filed as Exhibit 4.2 on the Company's Form 1012G filed on July 28, 2016.
4.3	Description of Securities of Mustang Bio, Inc. **
10.1	Second Amended and Restated Founders Agreement between Fortress Biotech, Inc. and Mustang Bio, Inc., dated July 26, 2016. * Filed as Exhibit 10.1 on the Company's Form 1012G filed on July 28, 2016.
10.2	Management Services Agreement between Fortress Biotech, Inc. and Mustang Bio, Inc., dated March 13, 2015. * Filed as Exhibit 10.2 on the Company's Form 1012G filed on July 28, 2016.
10.3	Future Advance Promissory Note to Fortress Biotech, Inc., dated May 5, 2016. * Filed as Exhibit 10.3 on the Company's Form 1012G filed on July 28, 2016.
10.4	Promissory Note to NSC Biotech Venture Fund I, LLC, dated July 5, 2016. * Filed as Exhibit 10.4 on the Company's Form 1012G filed on July 28, 2016.
10.5	Common Stock Warrant issued by Mustang Bio, Inc. to NSC Biotech Venture Fund I, LLC, dated July 5, 2016. * Filed as Exhibit 10.5 on the Company's Form 1012G filed on July 28, 2016.
10.6	License Agreement by and between Mustang Bio, Inc. and City of Hope, dated March 17, 2015. # Filed as Exhibit 10.6 on the Company's Form 1012G filed on July 28, 2016.
10.7	Sponsored Research Agreement by and between Mustang Bio, Inc. and City of Hope, dated March 17, 2015. * Filed as Exhibit 10.7 on the Company's Form 1012G filed on July 28, 2016.

Exhibit No.	Description
10.8	Mustang Bio, Inc. 2016 Incentive Plan. †* Filed as Exhibit 10.8 on the Company's Form 1012G filed on July 28, 2016.
10.9	Non-Employee Directors Compensation Plan. †* Filed as Exhibit 10.9 on the Company's Form 1012G filed on July 28, 2016.
10.10	Agreement with Chord Advisors, LLC, dated April 8, 2016. * Filed as Exhibit 10.10 on the Company's Form 1012G filed on July 28, 2016.
10.11	Agreement with Caribe BioAdvisors, LLC, dated January 1, 2017. Filed as Exhibit 10.11 on the Company's Form 10K filed on March 31, 2017.
10.12	Exclusive License Agreement with The Regents of the University of California, dated March 17, 2017. # Filed as Exhibit 10.4 on the Company's Form 10Q filed on August 14, 2017.
10.13	Exclusive License Agreement - IV/ICV with City of Hope, dated February 17, 2017. # Filed as Exhibit 10.5 on the Company's Form 10Q filed on August 14, 2017.
10.14	Amended and Restated Exclusive License Agreement - CD123 with City of Hope, dated February 17, 2017. # Filed as Exhibit 10.14 on the Company's Form 10K filed on March 31, 2017.
10.15	Amended and Restated Exclusive License Agreement - *IL13Ra2* with City of Hope, dated February 17, 2017. # Filed as Exhibit 10.15 on the Company's Form 10K filed on March 31, 2017.
10.16	Amended and Restated Exclusive License Agreement - Spacer with City of Hope, dated February 17, 2017. # Filed as Exhibit 10.16 on the Company's Form 10K filed on March 31, 2017.
10.17	Employment Agreement between Manuel Litchman and Mustang Bio, Inc., made effective as of April 24, 2017 Filed as Exhibit 10.1 on the Company's Form 8K filed on April 24, 2017.
10.18	License Agreement dated May 31, 2017 by and between Mustang Bio, Inc. and City of Hope (CSI). # Filed as Exhibit 10.1 on the Company's Form 10Q/A filed on November 14, 2017.
10.19	License Agreement dated May 31, 2017 by and between Mustang Bio, Inc. and City of Hope (PSCA). # Filed as Exhibit 10.2 on the Company's Form 10Q/A filed on November 14, 2017.
10.20	License Agreement dated May 31, 2017 by and between Mustang Bio, Inc. and City of Hope (HER2). # Filed as Exhibit 10.3 on the Company's Form 10Q/A filed on November 14, 2017.
10.21	Lease Agreement, by and between the Company and WCS - 377 Plantation Street, Inc., dated October 27, 2017. Filed as Exhibit 10.1 on the Company's Form 10Q filed on November 14, 2017.
10.22	Venture Loan and Security Agreement, dated March 29, 2019, by and between the Company and Horizon Technology Finance Corporation. * Filed as Exhibit 10.1 on the Company's Form 8K filed on April 4, 2019.
10.23	Mustang Bio, Inc. 2019 Employee Stock Purchase Plan†* Filed as Exhibit 10.1 on the Company's Form 10-Q filed on August 9, 2019.
10.24	Second Amendment to the Mustang Bio, Inc. 2016 Equity Incentive Plan. †* Filed as Exhibit 10.1 on the Company's Form 8-K filed on June 22, 2021.
10.25	Amendment to the Mustang Bio, Inc. 2019 Employee Stock Purchase Plan. †* Filed as Exhibit 10.2 on the Company's Form 8-K filed on June 22, 2021.

Exhibit No.	Description
16.1	Letter from BDO USA, LLP to the Securities and Exchange Commission dated September 22, 2021, incorporated by reference to the Form 8-K filed on September 24, 2021. *
23.1	Consent of Independent Registered Public Accounting Firm, BDO USA, LLP, Boston, Massachusetts.
23.2	Consent of Independent Registered Public Accounting Firm, KPMG, LLP, Hartford, Connecticut.
24.1	Power of Attorney (included on signature page).
31.1	Certification of President and Chief Executive Officer, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Principal Financial Officer, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of President and Chief Executive Officer, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2	Certification of Principal Financial Officer, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101	The following financial information from Mustang Bio, Inc.'s Annual Report on Form 10-K for the year ended December 31, 2021, formatted in Inline Extensible Business Reporting Language (iXBRL): (i) the Balance Sheets, (ii) the Statements of Operations, (iii) the Statement of Stockholders' Equity, (iv) the Statements of Cash Flows, and (v) Notes to the Financial Statements (filed herewith).
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in exhibit 101)

Confidential treatment has been granted with respect to omitted portions of this exhibit.

† Indicates management contract or compensatory plan or arrangement.

* Previously Filed.

** Filed herewith.

Item 16. Form 10-K Summary.

None.

INDEX TO FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors
Mustang Bio, Inc.
Worcester, Massachusetts

Opinion on the Financial Statements
We have audited the accompanying balance sheet of Mustang Bio, Inc. (the Company) as of December 31, 2021, the related statements of operations, stockholders' equity, and cash flows for the year then ended, and the related notes (collectively, the financial statements). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2021, and the results of its operations and its cash flows for the year then ended, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion
These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audit, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audit included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audit provides a reasonable basis for our opinion.

/s/ KPMG LLP

We have served as the Company's auditor since 2021.

Hartford, Connecticut
March 23, 2022

Report of Independent Registered Public Accounting Firm

Shareholders and Board of Directors
Mustang Bio, Inc.
Worcester, Massachusetts

Opinion on the Financial Statements

We have audited the accompanying balance sheet of Mustang Bio, Inc. (the "Company") as of December 31, 2020, the related statements of operations, stockholders' equity, and cash flows for the year ended December 31, 2020, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2020, and the results of its operations and its cash flows for the year ended December 31, 2020, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) ("PCAOB") and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audit we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audit included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audit provides a reasonable basis for our opinion.

/s/ BDO USA, LLP

Boston, Massachusetts
March 24, 2021

We have served as the Company's auditor from 2016 to 2021.

MUSTANG BIO, INC.
BALANCE SHEETS
(in thousands, except for share and per share amounts)

	December 31, 2021	December 31, 2020
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 109,618	$ 97,804
Other receivables - related party	50	15
Prepaid expenses and other current assets	2,038	1,715
Total current assets	111,706	99,534
Property, plant and equipment, net	9,025	7,529
Fixed assets - construction in process	2,027	499
Restricted cash	1,000	1,000
Other assets	362	250
Operating lease right-of-use asset, net	1,050	1,088
Total Assets	**$ 125,170**	**$ 109,900**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current Liabilities:		
Accounts payable and accrued expenses	$ 9,744	$ 8,747
Payables and accrued expenses - related party	723	490
Operating lease liabilities - short-term	348	278
Total current liabilities	**10,815**	**9,515**
Deferred income	270	—
Operating lease liabilities - long-term	1,685	1,950
Total Liabilities	**12,770**	**11,465**
Commitments and Contingencies		
Stockholders' Equity		
Preferred stock ($0.0001 par value), 2,000,000 shares authorized, 250,000 shares of Class A preferred stock issued and outstanding as of December 31, 2021 and 2020, respectively	—	—
Common Stock ($0.0001 par value), 150,000,000 and 125,000,000 shares authorized as of December 31, 2021 and 2020, respectively		
Class A common shares, 845,385 shares issued and outstanding as of December 31, 2021 and 2020, respectively	—	—
Common shares, 93,582,991 and 70,920,693 shares issued and outstanding as of December 31, 2021 and 2020, respectively	9	7
Common stock issuable, 2,536,607 and 2,103,122 shares as of December 31, 2021 and 2020, respectively	4,329	7,939
Additional paid-in capital	359,906	275,963
Accumulated deficit	(251,844)	(185,474)
Total Stockholders' Equity	**112,400**	**98,435**
Total Liabilities and Stockholders' Equity	**$ 125,170**	**$ 109,900**

See accompanying notes to financial statements.

MUSTANG BIO, INC.
STATEMENTS OF OPERATIONS
(in thousands, except for share and per share amounts)

	For the year ended December 31,	
	2021	2020
Operating expenses:		
Research and development	$ 49,864	$ 37,237
Research and development – licenses acquired	5,842	10,064
General and administrative	11,017	9,505
Total operating expenses	66,723	56,806
Loss from operations	(66,723)	(56,806)
Other income (expense)		
Interest income	368	708
Interest expense	(15)	(3,917)
Total other income (expense)	353	(3,209)
Net Loss	**$ (66,370)**	**$ (60,015)**
Net loss per common share outstanding, basic and diluted	$ (0.76)	$ (1.14)
Weighted average number of common shares outstanding, basic and diluted	87,885,235	52,588,781

See accompanying notes to financial statements.

MUSTANG BIO, INC.
STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands, except share amounts)

	Class A Preferred Stock		Class A Common Shares		Common Shares		Common Stock Issuable	Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount				
Balances at December 31, 2019	**250,000**	**$ —**	**845,385**	**$ —**	**39,403,519**	**$ 4**	**$ 4,923**	**$ 172,184**	**$ (125,459)**	**$ 51,652**
Common stock issuable - Founders Agreement	—	—	—	—	—	—	7,577	—	—	**7,577**
Issuance of common shares - Founders Agreement	—	—	—	—	1,206,667	—	(4,923)	4,923	—	**—**
Issuance of common shares, net of offering costs - At-the-Market Offering	—	—	—	—	17,579,097	2	—	58,595	—	**58,597**
Issuance of common shares - Equity fee on At-the-Market Offering	—	—	—	—	342,773	—	362	1,152	—	**1,514**
Issuance of common shares, net of offering costs - Public Offering	—	—	—	—	11,455,604	1	—	34,841	—	**34,842**
Issuance of common shares - Equity fee on Public Offering	—	—	—	—	286,390	—	—	932	—	**932**
Issuance of common shares under ESPP	—	—	—	—	140,856	—	—	349	—	**349**
Stock-based compensation expenses	—	—	—	—	502,788	—	—	2,987	—	**2,987**
Exercise of warrants	—	—	—	—	2,999	—	—	—	—	**—**
Net loss	—	—	—	—	—	—	—	—	(60,015)	**(60,015)**
Balances at December 31, 2020	**250,000**	**$ —**	**845,385**	**$ —**	**70,920,693**	**$ 7**	**$ 7,939**	**$ 275,963**	**$ (185,474)**	**$ 98,435**
Common stock issuable - Founders Agreement	—	—	—	—	—	—	4,212	—	—	**4,212**
Issuance of common shares - Founders Agreement	—	—	—	—	2,001,490	—	(7,577)	7,577	—	**—**
Issuance of common shares, net of offering costs - At-the-Market Offering	—	—	—	—	19,419,944	2	—	70,620	—	**70,622**
Issuance of common shares - Equity fee on At-the-Market Offering	—	—	—	—	576,157	—	(245)	2,129	—	**1,884**
Issuance of common shares under ESPP	—	—	—	—	114,321	—	—	309	—	**309**
Correction to previously issued shares	—	—	—	—	60,999	—	—	—	—	**—**
Stock-based compensation expenses	—	—	—	—	489,249	—	—	3,308	—	**3,308**
Exercise of warrants	—	—	—	—	138	—	—	—	—	**—**
Net loss	—	—	—	—	—	—	—	—	(66,370)	**(66,370)**
Balances at December 31, 2021	**250,000**	**$ —**	**845,385**	**$ —**	**93,582,991**	**$ 9**	**$ 4,329**	**$ 359,906**	**$ (251,844)**	**$ 112,400**

See accompanying notes to financial statements.

MUSTANG BIO, INC.
STATEMENTS OF CASH FLOWS
(in thousands)

	For the year ended December 31,	
	2021	**2020**
Cash Flows from Operating Activities:		
Net loss	$ (66,370)	$ (60,015)
Adjustments to reconcile net loss to net cash used in operating activities:		
Issuance of common shares - Equity fee on At-the-Market Offering to Fortress Biotech	1,884	1,514
Issuance of common shares - Equity fee on Public Offering to Fortress Biotech	—	932
Common shares issuable for Founders Agreement	4,212	7,577
Research and development - licenses acquired	1,630	2,487
Stock-based compensation expenses	3,308	2,987
Depreciation expense	2,167	1,677
Accretion of debt discount	—	2,321
Amortization of operating lease right-of-use assets	139	108
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(401)	(84)
Other receivables - related party	(35)	4
Accounts payable and accrued expenses	(408)	3,151
Payable and accrued expenses - related party	233	(106)
Deferred income	270	—
Lease liabilities	(296)	128
Net cash used in operating activities	(53,667)	(37,319)
Cash Flows from Investing Activities:		
Purchase of research and development licenses	(1,380)	(2,487)
Purchase of fixed assets	(3,986)	(1,925)
Net cash used in investing activities	(5,366)	(4,412)
Cash Flows from Financing Activities:		
Proceeds from issuance of common shares - At-the-Market Offering	71,919	59,805
Offering costs for the issuance of common shares - At-the-Market Offering	(1,381)	(1,124)
Proceeds from issuance of common shares - Public Offering	—	37,230
Offering costs for the issuance of common shares - Public Offering	—	(2,388)
Repayment of notes payable	—	(15,750)
Proceeds from issuance of common shares under ESPP	309	349
Net cash provided by financing activities	70,847	78,122
Net change in cash, cash equivalents and restricted cash	11,814	36,391
Cash, cash equivalents and restricted cash, beginning of the period	98,804	62,413
Cash, cash equivalents and restricted cash, end of the period	**$ 110,618**	**$ 98,804**
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ —	$ 1,593
Supplemental disclosure of noncash investing and financing activities:		
Fixed assets (acquired but not paid)	$ 1,270	$ 31
Issuance of common shares - Founders Agreement	$ 7,577	$ 4,923
Research and development licenses included in accounts payable and accrued expenses	$ 250	$ —
Lease liabilities arising from obtaining right-of-use assets	$ 101	$ —
Offering costs for the issuance of common shares - Public Offering, in accounts payable and accrued expenses	$ —	$ 84

See accompanying notes to financial statements.

Notes to Financial Statements

Note 1 - Organization and Description of Business

Mustang Bio, Inc. (the “Company” or “Mustang”) was incorporated in Delaware on March 13, 2015. Mustang is as a clinical-stage biopharmaceutical company focused on translating today’s medical breakthroughs in cell and gene therapy into potential cures for hematologic cancers, solid tumors and rare genetic diseases. The Company may acquire rights to these technologies by licensing the rights or otherwise acquiring an ownership interest in the technologies, funding their research and development and eventually either out-licensing or bringing the technologies to market.

The Company is a majority-controlled subsidiary of Fortress Biotech, Inc. (“Fortress” or “Parent”).

Liquidity and Capital Resources

The Company has incurred substantial operating losses and expects to continue to incur significant operating losses for the foreseeable future and may never become profitable. As of December 31, 2021, the Company had an accumulated deficit of $251.8 million.

The Company has funded its operations to date primarily through the sale of equity, its Loan Agreement with Runway and its venture debt financing agreement (the "Horizon Loan Agreement") with Horizon Technology Finance Corporation ("Horizon"), herein referred to as the "Horizon Notes." In September 2020, we repaid the Horizon Notes in full all amounts that were outstanding under the Horizon Loan Agreement, which was comprised of $15.0 million face value of the outstanding notes, $112,500 accrued and unpaid interest, a $750,000 loan termination fee and prepayment penalties of $550,000. The Company expects to continue to use the proceeds from previous financing transactions primarily for general corporate purposes, including financing the Company’s growth, developing new or existing product candidates, and funding capital expenditures, acquisitions and investments. The Company currently anticipates that its cash and cash equivalents balances at December 31, 2021, are sufficient to fund its anticipated operating cash requirements for at least one year from the date of this Form 10-K.

The Company will be required to expend significant funds in order to advance the development of its product candidates. The Company will require additional financings through equity and debt offerings, collaborations and licensing arrangements or other sources to fully develop, prepare regulatory filings, obtain regulatory approvals and commercialize its existing and any new product candidates. In addition to the foregoing, based on the Company’s current assessment, the Company does not expect any material impact on its long-term development timeline and its liquidity due to the worldwide spread of the COVID-19 virus. However, the Company is continuing to assess the effect on its operations by monitoring the impact of COVID-19 and the actions implemented to combat the virus throughout the world.

Note 2 - Significant Accounting Policies

Basis of Presentation

The Company’s financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (“GAAP”). The Company has no subsidiaries.

All inter-company transactions between Fortress and Mustang are classified as due from or due to related party in the financial statements. The Company believes that the assumptions underlying the financial statements are reasonable.

Segments

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating and reporting segment.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Actual results could differ from those estimates.

Cash and Cash Equivalents

The Company considers all short-term investments with an original maturity of three months or less when purchased to be cash equivalents. Cash and cash equivalents at December 31, 2021 and 2020, consisted of cash and money market funds in institutions in the United States. Balances at certain institutions have exceeded Federal Deposit Insurance Corporation insured limits.

Other Receivables – Related Party

Other receivables includes amounts due to the Company from Fortress and Journey Medical Corporation, both related parties, and is recorded at the invoiced amount.

Restricted Cash

The Company records cash held in an escrow account as a security deposit for the manufacturing facility in Worcester, Massachusetts, as restricted cash. The Company had $1.0 million in restricted cash as of December 31, 2021 and 2020, respectively. The Facility initiated cell processing operations for personalized CAR T and gene therapies in 2018.

Property, plant and equipment, net

Property and equipment, net, which consists mainly of laboratory equipment, are carried at cost less accumulated depreciation. Depreciation is computed over the estimated useful lives of the respective assets, generally five years, using the straight-line method.

Property and equipment - Construction in Process

In connection with the Company's cell processing facility, the Company incurred costs for the design and construction of the facility and the purchase of equipment; $2.0 million and $0.5 million are recorded in fixed assets - construction in process on the balance sheet at December 31, 2021 and 2020, respectively. Upon completion of the facility's construction, all costs associated with the buildout will be recorded as leasehold improvements and amortized over the shorter of the estimated useful lives or the term of the respective leases, upon the improvement being placed in service.

Research and Development Costs

Research and development costs are expensed as incurred. Advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made. Upfront and milestone payments due to third parties that perform research and development services on the Company's behalf will be expensed as services are rendered or when the milestone is achieved.

Research and development costs primarily consist of personnel related expenses, including salaries, benefits, travel, and other related expenses, stock-based compensation, payments made to third parties for license and milestone costs related to in-licensed products and technology, payments made to third party contract research organizations for preclinical and clinical studies, investigative sites for clinical trials, consultants, the cost of acquiring and manufacturing clinical trial materials, costs associated with regulatory filings, laboratory costs and other supplies.

In accordance with Accounting Standards Codification ("ASC") 730-10-25-1, *Research and Development,* costs incurred in obtaining technology licenses are charged to research and development expense if the technology licensed has not reached commercial feasibility and has no alternative future use. The licenses purchased by the Company require

substantial completion of research and development, regulatory and marketing approval efforts to reach commercial feasibility and has no alternative future use. Accordingly, the total purchase price for the licenses acquired is reflected as research and development - licenses acquired in the Company's Statements of Operations.

Annual Stock Dividend

In July 2016, in connection with the Amended and Restated Articles of Incorporation, the Company issued 250,000 Class A preferred shares to Fortress. The Class A preferred shares entitle the holder to a stock dividend equal to 2.5% of the fully diluted outstanding equity of the Company ("The Annual Stock Dividend"). The Annual Stock Dividend was part of the consideration payable for formation of the Company and the identification of certain assets, including the license contributed to Mustang by Fortress (see Note 4).

In June 2018, in connection with the Amended and Restated Articles of Incorporation, the Company amended the annual stock dividend due date from March 13th to January 1st.

Pursuant to the Amended and Restated Articles of Incorporation, the Company issued 2,536,607 shares of common stock to Fortress for the Annual Stock Dividend, representing 2.5% of the fully-diluted outstanding equity of Mustang on January 1, 2022. This was shown in the Statement of Stockholders' Equity at December 31, 2021, as Common stock issuable – Founders Agreement. The Company recorded an expense of approximately $4.2 million in research and development - licenses acquired related to these issuable shares during the year ended December 31, 2021.

Pursuant to the Amended and Restated Articles of Incorporation, the Company issued 2,001,490 shares of common stock to Fortress for the Annual Stock Dividend, representing 2.5% of the fully-diluted outstanding equity of Mustang on January 1, 2021. This was shown in the Statement of Stockholders' Equity at December 31, 2020 as Common stock issuable - Founders Agreement. The Company recorded an expense of approximately $7.6 million in research and development – licenses acquired related to these issuable shares during the year ended December 31, 2020.

Fair Value Measurement

The Company follows accounting guidance on fair value measurements for financial assets and liabilities measured at fair value on a recurring basis. Under the accounting guidance, fair value is defined as an exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or a liability.

The accounting guidance requires fair value measurements be classified and disclosed in one of the following three categories:

Level 1: Quoted prices in active markets for identical assets or liabilities.

Level 2: Observable inputs other than Level 1 prices, for similar assets or liabilities that are directly or indirectly observable in the marketplace.

Level 3: Unobservable inputs which are supported by little or no market activity and that are financial instruments whose values are determined using pricing models, discounted cash flow methodologies, or similar techniques, as well as instruments for which the determination of fair value requires significant judgment or estimation.

The fair value hierarchy also requires an entity to maximize the use of observable inputs and minimize the use of unobservable inputs when measuring fair value. Assets and liabilities measured at fair value are classified in their entirety based on the lowest level of input that is significant to the fair value measurement. The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires management to make judgments and consider factors specific to the asset or liability.

Leases

Arrangements meeting the definition of a lease are classified as operating or financing leases and are recorded on the balance sheet as both a right of use asset and lease liability, calculated by discounting fixed lease payments over the lease term at the rate implicit in the lease or the Company's incremental borrowing rate. Lease liabilities are increased by interest and reduced by payments each period, and the right of use asset is amortized over the lease term. For operating leases, interest on the lease liability and the amortization of the right of use asset result in straight-line rent expense over the lease term. Variable lease expenses are recorded when incurred. In calculating the right of use asset and lease liability, the Company elects to combine lease and non-lease components. The Company excludes short-term leases having initial terms of 12 months or less from the new guidance as an accounting policy election and recognizes rent expense on a straight-line basis over the lease term.

Stock-Based Compensation

The Company expenses stock-based compensation to employees over the requisite service period based on the estimated grant-date fair value of the awards and forfeiture rates.

The Company estimates the fair value of stock option grants using the Black-Scholes option pricing model or 409a valuations, as applicable. The assumptions used in calculating the fair value of stock-based awards represent management’s best estimates and involve inherent uncertainties and the application of management’s judgment.

Income Taxes

The Company records income taxes using the asset and liability method. Deferred income tax assets and liabilities are recognized for the future tax effects attributable to temporary differences between the financial statement carrying amounts of existing assets and liabilities and their respective income tax bases, and operating loss and tax credit carryforwards. The Company establishes a valuation allowance if management believes it is more likely than not that the deferred tax assets will not be recovered based on an evaluation of objective verifiable evidence. For tax positions that are more likely than not of being sustained upon audit, the Company recognizes the largest amount of the benefit that is greater than 50% likely of being realized. For tax positions that are not more likely than not of being sustained upon audit, the Company does not recognize any portion of the benefit.

Net Loss per Share

Net loss per share is computed by dividing net loss by the weighted average number of common shares outstanding during the period less unvested restricted stock. Since dividends are declared, paid and set aside among the holders of shares of common stock and Class A common shares pro-rata on an as-if-converted basis, the two-class method of computing net loss per share is not required. Diluted net loss per share does not reflect the effect of shares of common stock to be issued upon the exercise of warrants or outstanding Class A preferred shares, as their inclusion would be anti-dilutive.

The table below summarizes potentially dilutive securities that were not considered in the computation of diluted net loss per share because they would be anti-dilutive.

	For the year ended December 31,	
	2021	2020
Warrants	3,308,654	5,402,670
Options	1,141,675	1,141,675
Class A Preferred Shares	250,000	250,000
Unvested restricted stock awards	280,983	302,114
Unvested restricted stock units	2,335,557	1,468,559
Total	7,316,869	8,565,018

Comprehensive Loss

The Company has no components of other comprehensive loss, and therefore, comprehensive loss equals net loss.

Recent Accounting Pronouncements

In August 2020, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2020-06, *"Debt-Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging-Contracts in Entity's Own Equity (Subtopic 815-40): Accounting for Convertible Instruments and Contracts in an Entity's Own Equity,"* which simplifies accounting for convertible instruments by removing major separation models required under current GAAP. The ASU removes certain settlement conditions that are required for equity contracts to qualify for the derivative scope exception, and it also simplifies the diluted earnings per share calculation in certain areas. This guidance is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2023, including interim periods within those fiscal years. Early adoption will be permitted. The Company is currently evaluating the impact of this standard on its financial statements.

In June 2016, FASB issued ASU 2016-13, *"Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments"*. ASU 2016-13 requires that expected credit losses relating to financial assets are measured on an amortized cost basis and available-for-sale debt securities be recorded through an allowance for credit losses. ASU 2016-13 limits the amount of credit losses to be recognized for available-for-sale debt securities to the amount by which carrying value exceeds fair value and also requires the reversal of previously recognized credit losses if fair value increases. Recently, the FASB issued the final ASU to delay adoption for smaller reporting companies to calendar year 2023. The Company is currently assessing the impact of the adoption of this ASU on its financial statements.

Note 3 - License, Clinical Trial and Sponsored Research Agreements

Research and Development Expenses – All Licenses

For the years ended December 31, 2021 and 2020, the Company recorded the following expense in research and development for licenses acquired:

($ in thousands)	For the year ended December 31, 2021	2020
City of Hope National Medical Center		
CD123	$ 250	$ 334
CS1	—	200
IL13Rα2	—	333
Spacer	—	333
PSCA	250	200
HER2	—	500
CSL Behring (Calimmune)	30	170
Leiden University Medical Centre	350	—
Fred Hutchinson Cancer Research Center - CD20	—	300
Mayo Clinic	750	—
SIRION Biotech LentiBOOST™	—	117
Fortress PIK Dividend	4,212	7,577
Total	$ 5,842	$ 10,064

License Agreements

City of Hope

CD123 License (MB-102)

In February 2017, the Company entered into an Amended and Restated Exclusive License Agreement with the City of Hope National Medical Center ("COH") to acquire intellectual property rights pertaining to CD123 -specific chimeric antigen receptor ("CAR") engineered T cell ("CAR T") technology. Pursuant to this agreement, the Company and COH acknowledged that an upfront fee was previously paid. In addition, COH is eligible to receive an annual maintenance fee

of $25,000 and milestone payments totaling $14.5 million upon the achievement of certain milestones. Royalty payments in the mid-single digits are due on net sales of licensed products.

For the year ended December 31, 2021, the Company expensed a non-refundable milestone payment of $0.3 million for the 24th patient treated in the Phase 1 clinical study for MB-102 at COH. For the year ended December 31, 2020, the Company expensed a non-refundable milestone payment of $0.3 million in connection with Mustang's public underwritten offerings.

CS1 License (MB-104)

In May 2017, the Company entered into an exclusive license agreement with the COH for the use of CS1-specific CAR T technology. Pursuant to this agreement, the Company paid an upfront fee of $0.6 million and pays an annual maintenance fee of $50,000. Additional payments are due for the achievement of ten development milestones totaling $14.9 million, and royalty payments in the mid-single digits are due on net sales of licensed products.

For the year ended December 31, 2020, the Company expensed a non-refundable milestone payment of $0.2 million upon issuance of the first patent related to the CS1 CAR T technology.

IL13Rα2 License (MB-101)

In February 2017, the Company entered into an Amended and Restated Exclusive License Agreement with COH to acquire intellectual property rights pertaining to IL13Rα2-specific CAR T technology. Pursuant to this agreement, payments are due for the achievement of eight development milestones totaling $14.5 million; additional payments are due upon the occurrence of certain one-time; events and royalty payments as a percentage of revenue in the mid-single digits are due on net sales of licensed products.

For the year ended, December 31, 2020, the Company expensed a non-refundable milestone payment of $0.3 million in connection with Mustang's public underwritten offerings.

Spacer License

In February 2017, the Company entered into an Amended and Restated Exclusive License Agreement with COH to acquire intellectual property rights pertaining to Spacer patent rights. Pursuant to this agreement, payments are due upon the occurrence of certain one-time events and royalty payments as a percentage of revenue in the low single digits are due on net sales of licensed products.

For the year ended December 31, 2020, the Company expensed a non-refundable milestone payment of $0.3 million in connection with Mustang's public underwritten offerings.

PSCA License (MB-105)

In May 2017, the Company entered into an exclusive license agreement with COH for the use of prostate stem cell antigen ("PSCA") CAR T technology to be used in the treatment of prostate cancer, pancreatic cancer and other solid tumors. Pursuant to this agreement, the Company paid an upfront fee of $0.3 million and pays an annual maintenance fee of $50,000. Additional payments are due for the achievement of ten development milestones totaling $14.9 million, and royalty payments in the mid-single digits are due on net sales of licensed products.

For the year ended December 31, 2021, the Company expensed a non-refundable milestone payment of $0.3 million for the twelfth patient treated in the Phase 1 clinical study of MB-105 at COH. For the year ended December 31, 2020, the Company expensed a non-refundable milestone payment of $0.2 million upon issuance of the first patent related to the PCSA CAR T technology.

HER2 License (MB-103)

On May 31, 2017, the Company entered into an exclusive license agreement with the COH for the use of human epidermal growth factor receptor 2 ("HER2") CAR T technology, which will initially be applied in the treatment of glioblastoma

multiforme. Pursuant to this agreement, the Company paid an upfront fee of $0.6 million and pays an annual maintenance fee of $50,000 (which began in 2019). Additional payments are due for the achievement of ten development milestones totaling $14.9 million, and royalty payments in the mid-single digits are due on net sales of licensed products.

For the year ended, December 31, 2020, the Company expensed a non-refundable milestone payment of $0.5 million in connection with the twelfth patient treated in the Phase 1 clinical study of HER2 CAR T technology at COH.

CSL Behring (Calimmune) License

On August 23, 2019, the Company entered into a non-exclusive license agreement with CSL Behring (Calimmune, Inc.) ("Calimmune License") for the rights to the Cytegrity™ stable producer cell line for the production of viral vector for our lentiviral gene therapy program for the treatment of XSCID (MB-107 and MB-207). We previously licensed the XSCID gene therapy program from St. Jude Children's Research Hospital, Inc. ("St. Jude") in August 2018. Pursuant to the terms of the Calimmune License, the Company paid an upfront fee of $0.2 million. CSL Behring is eligible to receive additional payments totaling $1.2 million upon the achievement of three development and commercialization milestones. Royalty payments in the low-single digits are due on net sales of licensed products.

For the year ended December 31, 2021 and 2020, the Company expensed a non-refundable milestone payments of $30,000 and $0.2 million, respectively, in connection with the Calimmune license.

Leiden University Medical Centre License (MB-110)

On September 8, 2021, the Company entered into an exclusive, worldwide licensing agreement with Leiden University Medical Centre ("Leiden") for the use of a gene therapy under development for the treatment of severe immunodeficiency caused by RAG1 deficiency (the "Leiden License"). Pursuant to the Leiden License, the Company expensed an upfront fee of $0.4 million. Additional payments are due for the achievement of certain development milestones totaling up to $31 million and royalty payments in the low to mid-single digits as a percentage of revenue are due on net sales of licensed products.

For the year ended December 31, 2021, the Company expensed an upfront payment of $0.4 million in connection with the Leiden License.

Fred Hutchinson Cancer Research Center - CD20 License (MB-106)

On July 3, 2017, the Company entered into an exclusive, worldwide licensing agreement with Fred Hutchinson Cancer Research Center ("Fred Hutch") for the use of a CAR T therapy related to autologous T cells engineered to express a CD20-specific chimeric antigen receptor ("CD20 Technology License"). Pursuant to the CD20 Technology License, the Company paid Fred Hutch an upfront fee of $0.3 million and will owe an annual maintenance fee of $50,000 on each anniversary of the license until the achievement by the Company of regulatory approval of a licensed product using CD20 Technology. Additional payments are due for the achievement of eleven development milestones totaling $39.1 million and royalty payments in the mid-single digits are due on net sales of licensed products.

For the year ended December 31, 2020, the Company expensed a non-refundable milestone payment of $0.3 million in connection with the twelfth patient treated in the Phase 1 clinical study of CD20 CAR T technology at Fred Hutch.

Mayo Clinic - CAR T Technology License

On April 1, 2021, the Company entered into an exclusive license agreement with the Mayo Foundation for Medical Education and Research (the "Mayo Clinic") for a novel technology that may be able to transform the administration of CAR T therapies and has the potential to be used as an off-the shelf therapy. Pursuant to this agreement, the Company paid an upfront fee of $0.8 million and will pay an annual maintenance fee of $25,000. Additional payments are due for each of two licensed products for the achievement of eleven development and commercial milestones totaling up to $92.6 million per product, and royalty payments in the mid-single digits as a percentage of revenue are due on net sales of licensed products.

For the year ended December 31, 2021, the Company expensed an upfront payment of $0.8 million pursuant to the terms of the license agreement.

SIRION Biotech GmbH - LentiBOOST™

In October 2020, we announced a licensing agreement with SIRION Biotech ("SIRION") for the rights to SIRION's LentiBOOST™ technology for the development of MB-207, our lentiviral gene therapy for the treatment of previously transplanted XSCID patients (the "SIRION Technology License"). Pursuant to the SIRION Technology License, the Company paid SIRION a one-time upfront fee of $0.1 million (€0.1 million). In addition, five future development milestone payments totaling up to approximately $5.2 million (€4.7 million) in the aggregate are due upon achievement of certain milestones. Additional milestone payments totaling up to $3.9 million (€3.5 million) in the aggregate are due in connection with the achievement of three commercial milestones and low- to mid-single digit royalties are due on aggregate cumulative worldwide net sales of licensed products.

In December 2021 this licensing agreement was amended to include CD20-directed CAR Ts. SIRION is eligible to receive additional payments totaling up to approximately $9.1 million upon the achievement of certain development and commercialization milestones for the additional product.

For the year ended December 31, 2020, the Company expensed an up-front payment of $0.1 million pursuant to the terms of the license agreement.

Research and Development Expenses - Sponsored Research and Clinical Trial Agreements

For the year ended December 31, 2021 and 2020, the Company recorded the following expense in research and development for sponsored research and clinical trial agreements:

($ in thousands)	For the year ended December 31, 2021	2020
City of Hope National Medical Center	$ —	$ 500
CD123	301	433
IL13Rα2	1,169	530
CS1	608	885
HER2	697	1,519
PSCA	107	204
Fred Hutchinson Cancer Research Center - CD20	1,979	1,804
St. Jude Children's Research Hospital - XSCID	865	1,842
LUMC - RAGI SCID	170	—
Mayo Clinic	695	—
Total	$ 6,591	$ 7,717

City of Hope

Sponsored Research Agreement

In March 2015, the Company entered into a Sponsored Research Agreement ("SRA") with COH in which the Company funded continued research in the amount of $2.0 million per year, payable in four equal installments, through the first quarter of 2020. The research covered under this arrangement is for IL13Rα2, CD123 and the Spacer technology. For the year ended December 31, 2020, the Company recorded $0.5 million in research and development expenses in the Statements of Operations pursuant to the terms of this agreement.

CD123 (MB-102) Clinical Research Support Agreement

In February 2017, the Company entered into a Clinical Research Support Agreement for CD123 (the "CD123 CRA"). Pursuant to the terms of the CD123 CRA the Company made an upfront payment of $19,450 and will contribute an additional $97,490 per patient in connection with the on-going investigator-initiated study. Further, the Company agreed

to fund approximately $0.2 million over three years pertaining to the clinical development of CD123. For the years ended December 31, 2021 and 2020, the Company recorded $0.3 million and $0.4 million, respectively, in research and development expenses in the Statements of Operations pursuant to the terms of this agreement.

IL13Rα2 (MB-101) Clinical Research Support Agreements

In February 2017, the Company entered into a clinical research support agreement for IL13Rα2 (the "IL13Rα2 CRA"). Pursuant to the terms of the IL13Rα2 CRA the Company made an upfront payment of approximately $9,300 and will contribute an additional $0.1 million related to patient costs in connection with the on-going investigator-initiated study. Further, the Company agreed to fund approximately $0.2 million over three years pertaining to the clinical development of IL13Rα2. For the years ended December 31, 2021 and 2020, the Company recorded $1.2 million and $0.5 million, respectively, in research and development expenses under the IL13Rα2 CRA in the Statements of Operations pursuant to the terms of this agreement.

In October 2020, the Company entered into a Clinical Research Support Agreement for the IL13Rα2-directed CAR T program for adult patients with leptomeningeal glioblastoma, ependymoma or medulloblastoma (the "IL13Rα2 Leptomeningeal CRA"). Pursuant to the terms of the IL13Rα2 Leptomeningeal CRA, the Company made an upfront payment of approximately $29,000 and will contribute an additional $0.1 million per patient in connection with the on-going investigator-initiated study. Further, the Company agreed to fund approximately $0.2 million annually pertaining to the clinical development of the IL13Rα2-directed CAR T therapy.

In March 2021, the Company entered into a clinical research support agreement for an Institutional Review Board-approved, investigator-initiated protocol entitled: "Single Patient Treatment with Intraventricular Infusions of IL13Rα2-targeting and HER2-targeting Chimeric Antigen Receptor (CAR)-T cells for a Single Patient (UPN 181) with Recurrent Multifocal Malignant Glioma." Pursuant to the terms of this agreement, the Company will contribute up to $0.2 million in connection with the ongoing investigator-initiated study.

CS1 (MB-104) Clinical Research Support Agreement

In June 2020, the Company entered into a clinical research support agreement with COH in connection with an Investigator-sponsored study conducted under an Institutional Review Board-approved, investigator-initiated protocol entitled: "Phase I Study to Evaluate Cellular Immunotherapy Using Memory-Enriched T Cells Lentivirally Transduced to Express a CS1-Targeting, Hinge-Optimized, 41BB-Costimulatory Chimeric Antigen Receptor and a Truncated EGFR Following Lymphodepleting Chemotherapy in Adult Patients with CS1+ Multiple Myeloma." The CAR T being studied under this protocol has been designated as MB-104. Under the terms of the agreement the Company will reimburse COH for costs associated with this trial not to exceed $2.4 million. The agreement will expire upon the delivery of a final study report or earlier. For the years ended December 31, 2021 and 2020, the Company recorded $0.6 million and $0.9 million, respectively, in research and development expenses in the Statements of Operations pursuant to the terms of this agreement.

HER2 (MB-103) Clinical Research Support Agreement

In September 2020, the Company entered into a clinical research support agreement with COH in connection with an Investigator-sponsored study conducted under an Institutional Review Board-approved, investigator-initiated protocol entitled: "Phase I Study of Cellular Immunotherapy using Memory-Enriched T Cells Lentivirally Transduced to Express a HER2-Specific, Hinge-Optimized, 41BB-Costimulatory Chimeric Receptor and a Truncated CD19 for Patients with Recurrent/Refractory Malignant Glioma." The CAR T being studied under this protocol has been designated as MB-103. Under the terms of the agreement the Company will pay COH $29,375 upon execution and will reimburse COH for costs associated with this trial not to exceed $3.0 million. The agreement will expire upon the delivery of a final study report or earlier. For the year ended December 31, 2021 and 2020, the Company recorded $0.7 million and $1.5 million, respectively, in research and development expenses in the Statements of Operations pursuant to the terms of this agreement.

PSCA (MB-105) Clinical Research Support Agreement

In October 2020, the Company entered into a clinical research support agreement with COH in connection with an Investigator-sponsored study conducted under an Institutional Review Board-approved, investigator-initiated protocol entitled: "A Phase 1b study to evaluate PSCA-specific chimeric antigen receptor (CAR)-T cells for patients with metastatic castration resistant prostate cancer." The CAR T being studied under this protocol has been designated as MB-105. Under the terms of the agreement the Company will pay COH $33,000 upon execution and will reimburse COH for costs associated with this trial not to exceed $2.3 million. The agreement will expire upon the delivery of a final study report or earlier. For the years ended December 31, 2021 and 2020, the Company recorded $0.1 million and $0.2 million, respectively, in research and development expenses in the Statements of Operations pursuant to the terms of this agreement.

Fred Hutchinson Cancer Research Center

CD20 Clinical Trial Agreement

On July 3, 2017, in conjunction with the CD20 Technology License from Fred Hutch, we entered into an investigator-initiated clinical trial agreement ("CD20 CTA") to provide partial funding for a Phase 1/2 clinical trial at Fred Hutch evaluating the safety and efficacy of the CD20 Technology in patients with relapsed or refractory B-cell non-Hodgkin lymphomas. In connection with the CD20 CTA, the Company agreed to fund up to $5.3 million of costs associated with the clinical trial, which commenced during the fourth quarter of 2017. In November 2020, the CD20 CTA was amended to include additional funding of approximately $1.8 million for the treatment of five patients with chronic lymphocytic leukemia and other research costs.

For the years ended December 31, 2021 and 2020, the Company recorded $2.0 million and $1.8 million, respectively, in research and development expenses in the Statements of Operations pursuant to the terms of this agreement.

XSCID (MB-107) Data Transfer Agreement with St. Jude Children's Research Hospital

In June 2020, the Company entered into a Data Transfer Agreement with St. Jude under which we will reimburse St. Jude for costs associated with St. Jude's clinical trial for the treatment of infants with XSCID. Pursuant to the terms of this agreement the Company paid an upfront fee of $1.1 million in July 2020, and will continue to reimburse St. Jude for costs incurred in connection with this clinical trial. For the years ended December 31, 2021 and 2020, the Company recorded $0.9 million and $1.8 million, respectively, in research and development expenses in the Statements of Operations pursuant to the terms of this agreement.

RAG1-SCID (MB-110) Sponsored Research Support Agreement with Leiden University Medical Centre

On September 8, 2021, in connection with the Leiden License, the Company entered into an SRA with Leiden under which the Company will fund research in the amount of approximately $0.5 million annually over a period of 5 years. The research performed pursuant to this agreement will support technology the Company has licensed from Leiden for the use of a gene therapy under development for the treatment of severe immunodeficiency caused by RAG1. For the year ended December 31, 2021, the Company recorded $0.2 million in research and development expenses in the Statements of Operations pursuant to the terms of this agreement.

Sponsored Research Support Agreement with Mayo Clinic

In June 2021, the Company entered into an SRA with Mayo Clinic under which the Company will fund research in the amount of $2.1 million over a period of two years. The research performed pursuant to this agreement will support technology the Company has licensed from Mayo Clinic for a novel technology that may be able to transform the administration of CAR T therapies and has the potential to be used as an off-the-shelf therapy. For the year ended December 31, 2021, the Company recorded $0.7 million in research and development expenses in the Statements of Operations pursuant to the terms of this agreement.

Note 4 - Related Party Agreements

Founders Agreement and Management Services Agreement with Fortress

Effective March 13, 2015, the Company entered a Founders Agreement with Fortress, which was amended and restated on May 17, 2016, and again on July 26, 2016 (the "Mustang Founders Agreement"). The Mustang Founders Agreement provides that, in exchange for the time and capital expended in the formation of Mustang and the identification of specific assets the acquisition of which result in the formation of a viable emerging growth life science company, Fortress loaned $2.0 million, representing the up-front fee required to acquire the Company's license agreement with COH. The Mustang Founders Agreement has a term of 15 years, which upon expiration automatically renews for successive one-year periods unless terminated by Fortress and the Company or a Change in Control (as defined in the Mustang Founders Agreement) occurs. Concurrently with the second amendment on July 26, 2016, to the Mustang Founders Agreement, Fortress entered into an Exchange Agreement whereby Fortress exchanged its 7.25 million Class B Common shares for 7.0 million common shares and 250,000 Class A Preferred shares. Class A Preferred Stock is identical to common stock other than as to voting rights, conversion rights and the PIK Dividend right (as described below). Each share of Class A Preferred Stock is entitled to vote the number of votes that is equal to one and one-tenth (1.1) times a fraction, the numerator of which is the sum of (A) the shares of outstanding Mustang common stock and (B) the whole shares of Mustang common stock into which the shares of outstanding Class A Common Stock and Class A Preferred Stock are convertible and the denominator of which is the number of shares of outstanding Class A Preferred Stock. Thus, the Class A Preferred Stock will at all times constitute a voting majority. Each share of Class A Preferred Stock is convertible, at Fortress' option, into one fully paid and nonassessable share of Mustang common stock, subject to certain adjustments. As holders of Class A Preferred Stock, Fortress will receive on each January 1 (each a "PIK Dividend Payment Date") until the date all outstanding Class A Preferred Stock is converted into common stock, pro rata per share dividends paid in additional fully paid and nonassessable shares of common stock ("PIK Dividends") such that the aggregate number of shares of common stock issued pursuant to such PIK Dividend is equal to two and one-half percent (2.5%) of Mustang's fully-diluted outstanding capitalization on the date that is one (1) business day prior to any PIK Dividend Payment Date.

As additional consideration under the Mustang Founders Agreement, Mustang will also: (i) pay an equity fee in shares of common stock, payable within five (5) business days of the closing of any equity or debt financing for Mustang that occurs after the effective date of the Mustang Founders Agreement and ending on the date when Fortress no longer has majority voting control in the Company's voting equity, equal to two and one-half (2.5%) of the gross amount of any such equity or debt financing; and (ii) pay a cash fee equal to four and one-half percent (4.5%) of the Company's annual net sales, payable on an annual basis, within ninety (90) days of the end of each calendar year. In the event of a Change in Control, the Company will pay a one-time change in control fee equal to five (5x) times the product of (A) net sales for the twelve (12) months immediately preceding the change in control and (B) four and one-half percent (4.5%) (see Note 9).

Effective as of March 13, 2015, the Company entered into a Management Services Agreement (the "MSA") with Fortress, pursuant to which Fortress renders advisory and consulting services to the Company. The MSA has an initial term of five years and is automatically renewed for successive five-year terms unless terminated in accordance with its provisions. Services provided under the MSA may include, without limitation, (i) advice and assistance concerning any and all aspects of the Company's operations, clinical trials, financial planning and strategic transactions and financings and (ii) conducting relations on behalf of the Company with accountants, attorneys, financial advisors and other professionals (collectively, the "Services"). The Company is obligated to utilize clinical research services, medical education, communication and marketing services and investor relations/public relation services of companies or individuals designated by Fortress, provided those services are offered at market prices. However, the Company is not obligated to take or act upon any advice rendered from Fortress and Fortress shall not be liable for any of its actions or inactions based upon their advice. Pursuant to the MSA and the Company's Certificate of Incorporation, Fortress and its affiliates, including all members of the Company's Board of Directors, will have no fiduciary or other duty to communicate or present any corporate opportunities to the Company or to refrain from engaging in business that is similar to that of the Company. In consideration for the Services, the Company will pay Fortress an annual consulting fee of $0.5 million (the "Annual Consulting Fee"), payable in advance in equal quarterly installments on the first business day of each calendar quarter in each year, provided, however, that such Annual Consulting Fee shall be increased to $1.0 million for each calendar year in which the Company has net assets in excess of $100 million at the beginning of the calendar year. The Company records fifty percent of the Annual Consulting Fee in research and development expense and fifty percent in general and administrative expense in

the Statement of Operations. For the years ended December 31, 2021 and 2020, the Company recorded expense of $0.5 million and $0.5 million, respectively, related to this agreement.

For the year ended December 31, 2021, the Company issued 576,157 shares of common stock and recorded 51,295 shares issuable to Fortress, which equaled 2.5% of the gross proceeds of $71.9 million from the sale of shares of common stock under Mustang's At-the-Market Offering. The Company recorded an expense of approximately $1.9 million in general and administrative expenses related to these shares for the year ended December 31, 2021.

For the year ended December 31, 2020, the Company issued 342,773 shares of common stock and recorded 101,632 shares issuable to Fortress, which equaled 2.5% of the gross proceeds of $59.8 million from the sale of shares of common stock under Mustang's At-the-Market Offering. The Company recorded an expense of approximately $1.5 million in general and administrative expenses related to these shares for the year ended December 31, 2020.

For the year ended December 31, 2020, the Company issued 286,390 shares of common stock to Fortress, which equaled 2.5% of the gross proceeds of $37.2 million from the sale of shares of common stock, before deducting underwriting discounts and commissions and offering expenses under Mustang's Public Offering. The Company recorded an expense of approximately $0.9 million in general and administrative expenses related to these shares during the year ended December 31, 2020.

Payables and Accrued Expenses Related Party

In the normal course of business Fortress pays for certain expenses on behalf of the Company. Such expenses are recorded as Payables and accrued expenses - related party and are reimbursed to Fortress in the normal course of business.

Director Compensation

Dr. Rosenwald

Pursuant to the terms of the Director Compensation Plan, Dr. Rosenwald will receive a cash fee of $50,000 per year paid quarterly and an annual stock award of the greater of (i) a number of shares of common stock having a fair market value on the grant date of $50,000 or (ii) 10,000 shares of common stock, which shares shall vest and become non-forfeitable on the third anniversary of the grant date, subject to continued service on the Board on such date.

For the year ended December 31, 2021, the Company recognized $106,000 in expense in its Statements of Operations related to the director compensation, including approximately $56,000 in expense related to equity incentive grants. For the year ended December 31, 2020, the Company recognized $113,000 in expense in its Statements of Operations related to the director compensation, including approximately $63,000 in expense related to equity incentive grants. The company issued Dr. Rosenwald 13,774 and 16,611 restricted stock awards for the years ended December 31, 2021 and 2020, respectively.

Mr. Weiss - Advisory Agreement with Caribe BioAdvisors, LLC

The Board of the Company by unanimous written consent approved and authorized the execution of an advisory agreement dated January 1, 2017 (the "Advisory Agreement"), with Caribe BioAdvisors, LLC (the "Advisor"), owned by Michael S. Weiss, the Chairman of the Board, to provide the board advisory services of Mr. Weiss as Chairman of the Board. Pursuant to the Advisory Agreement, the Advisor will be paid an annual cash fee of $60,000, paid quarterly and an annual stock award of the greater of (i) a number of shares of common stock having a fair market value on the grant date of $50,000 or (ii) 10,000 shares of common stock, which shares shall vest and become non-forfeitable on the third anniversary of the grant date, subject to continued service on the Board on such date.

For the year ended December 31, 2021, the Company recognized $116,000 in expense in its Statements of Operations related to the advisory agreement, including approximately $56,000 in expense related to equity incentive grants. For the year ended December 31, 2020, the Company recognized $122,000 in expense in its Statements of Operations related to the advisory agreement, including approximately $62,000 in expense related to equity incentive grants. The company issued Mr. Weiss 13,774 and 16,611 restricted stock awards for the years ended December 31, 2021 and 2020, respectively.

Note 5 - Property and Equipment

Mustang's property and equipment consisted of the following:

($ in thousands)	Estimated Useful Life (in years)	December 31, 2021	December 31, 2020
Computer equipment	3	$ 145	$ 68
Furniture and fixtures	5	370	181
Machinery and equipment	5	6,550	5,748
Leasehold improvements	9	7,694	5,099
Construction in process	N/A	2,027	499
Total property and equipment		16,786	11,595
Less: accumulated depreciation		(5,734)	(3,567)
Property and equipment, net		$ 11,052	$ 8,028

Mustang's depreciation expense for the years ended December 2021 and 2020 was approximately $2.2 million and $1.7 million, respectively, and was recorded in research and development expense in the Statements of Operations.

Note 6 - Accounts Payable and Accrued Expenses

At December 31, 2021 and 2020, accounts payable and accrued expenses consisted of the following:

($ in thousands)	December 31, 2021	December 31, 2020
Accounts payable	$ 3,512	$ 3,518
Research and development	3,083	2,862
Accrued compensation	2,595	2,009
Other	554	358
Total accounts payable and accrued expenses	$ 9,744	$ 8,747

Note 7 - Commitments and Contingencies

Leases

On October 27, 2017, the Company entered into a lease agreement with WCS - 377 Plantation Street, Inc., a Massachusetts nonprofit corporation. Pursuant to the terms of the lease agreement, the Company agreed to lease 27,043 square feet from the landlord, located at 377 Plantation Street in Worcester, MA (the "Facility"), through November 2026, subject to additional extensions at the Company's option. Base rent, net of abatements of $0.6 million over the lease term, totals approximately $3.6 million, on a triple-net basis.

The terms of the lease also require that the Company post an initial security deposit of $0.8 million, in the form of $0.5 million letter of credit and $0.3 million in cash, which increased to $1.3 million ($1.0 million letter of credit, $0.3 million in cash) on November 1, 2019. After the fifth lease year, the letter of credit obligation is subject to reduction.

The Facility began operations for the production of personalized CAR T and gene therapies in 2018.

The Company leases office space and copiers under agreements classified as operating leases that expire on various dates through 2026. The Company's lease liabilities result from the lease of its Facility in Massachusetts, which expires in 2026, and its copiers, which expire in 2024. Such leases do not require any contingent rental payments, impose any financial restrictions, or contain any residual value guarantees. Certain of the Company's leases include renewal options and escalation clauses; renewal options have not been included in the calculation of the lease liabilities and right of use assets as the Company is not reasonably certain to exercise the options. The Company does not act as a lessor or have any leases

classified as financing leases. At December 31, 2021, the Company had operating lease liabilities of $2.0 million and right of use assets of $1.1 million, which were included in the Balance Sheet. At December 31, 2020, the Company had operating lease liabilities of $2.2 million and right of use assets of $1.1 million, which were included in the Balance Sheet.

The following summarizes quantitative information about the Company's operating leases:

	For the Year Ended	
($ in thousands)	December 31, 2021	December 31, 2020
Lease cost		
Operating lease cost	$ 315	$ 330
Variable lease cost	599	470
Total	$ 914	$ 800

	For the Year Ended	
($ in thousands)	December 31, 2021	December 31, 2020
Operating cash flows from operating leases	$ 484	$ 76
Weighted-average remaining lease term – operating leases	4.8	5.8
Weighted-average discount rate – operating leases	9.0 %	9.0 %

Maturities of our operating leases, excluding short-term leases, are as follows:

($ in thousands)	
Year ended December 31, 2022	$ 516
Year ended December 31, 2023	529
Year ended December 31, 2024	513
Year ended December 31, 2025	516
Year ended December 31, 2026	439
Thereafter	—
Total	2,513
Less present value discount	(481)
Operating lease liabilities	$ 2,033

Note 8 – Notes Payable

On March 29, 2019 (the "Closing Date"), the Company entered into a $20.0 million Loan Agreement with Horizon, the proceeds of which provided the Company with additional working capital to continue development of its gene and cell therapies. In accordance with the Loan Agreement, $15.0 million of the $20.0 million loan was funded on the Closing Date, with the remaining $5.0 million fundable upon the Company achieving certain predetermined milestones.

Amortization of the debt discount associated with the funded loans was approximately $2.3 million for the year ended December 31, 2020, and was included in interest expense in the Statements of Operations.

On September 30, 2020, the Company repaid the amount outstanding under the Horizon Notes in full, which was comprised of $15.0 million face value of the outstanding notes, $0.1 million in accrued and unpaid interest, a $0.8 million final payment fee and prepayment penalties of $0.6 million. For the year ended December 31, 2020, the Company recorded interest expense of approximately $2.1 million related to the early repayment of the Horizon Notes.

Note 9 - Stockholders' Equity

Common Stock

The Company, in accordance with its certificate of incorporation, as amended in November 2020 and June 2021, which was retroactively applied, is authorized to issue (i) 150,000,000 common shares with a par value of $0.0001 per share, of which 1,000,000 shares are designated as Class A Common Stock and the remainder are undesignated Common Stock, and (ii) 2,000,000 shares of Preferred Stock, 250,000 of which are designated as Class A Preferred Stock and the remainder are undesignated Preferred Stock (see below Stock Issuances to Fortress and Note 4).

In connection with the Company's formation, Fortress subscribed for 7,000,000 shares of the Class B Common Stock and 2,000,000 shares of the Company's Common Stock, pursuant to the Founders Agreement. Fortress paid the par value of $900 in 2016. The fair value of the Company's common shares approximated par value as no licenses had been transferred at that time. Dividends, if and when declared, are to be distributed pro-rata to the Class A, B and Common Stockholders.

The holders of Common Stock are entitled to one vote per share of Common Stock held. The holders of Class A Common Stock are entitled to the number of votes equal to the number of whole shares of Common Stock into which the shares of Class A Common Stock held by such holder are convertible and for a period of ten years from its issuance, the holders of the Class A Common Stock have the right to appoint one member of the board of directors of Mustang; to date, the holders of Class A Common Stock have not yet appointed such director.

The Class B Common Stockholders are entitled, for each share of Class B Common Stock held, to a number of votes equal to 1.1 times a fraction, the numerator of which is the sum of (A) the shares of outstanding Common Stock and (B) the whole shares of Common Stock into which the shares of outstanding Class A Common Stock and the Class B Common Stock are convertible and the denominator of which is the number of shares of outstanding Class B common shares. There was no Class B Common Stock outstanding as of December 31, 2021.

On November 11, 2020, the Company's Board adopted resolutions of the Board to ratify, approve and recommend stockholder approval of an amendment to the Company's Amended and Restated Certificate of Incorporation, as amended, to revise Article IV, Section A thereof in order to effect an increase in the authorized number of shares of the Company's common stock, par value $0.0001, from 85,000,000 to 125,000,000 (the "Amendment"). On November 11, 2020, the Company received approval of the Amendment by written consent in lieu of a meeting from the holders of a majority of issued and outstanding shares of the Company's common and preferred stock. The increase in authorized shares to 125,000,000 became effective on December 4, 2020.

On June 17, 2021, the stockholders of the Company voted at the 2021 Annual Meeting to approve an amendment to Mustang's Amended and Restated Certificate of Incorporation to increase the number of shares of common stock authorized for issuance by 25,000,000 shares, bringing the total number of authorized shares of common stock to 150,000,000 shares. The increase in authorized shares to 150,000,000 became effective on June 17, 2021.

At-the-Market Offering of Common Stock

On July 13, 2018, the Company filed a shelf registration statement No. 333-226175 on Form S-3 , as amended on July 20, 2018 (the "2018 S-3"), which was declared effective in August 2018. Under the 2018 S-3, the Company may sell up to a total of $75.0 million of its securities. In connection with the 2018 S-3, the Company entered into an At-the-Market Issuance Sales Agreement (the "ATM Agreement") with B. Riley Securities, Inc. (formerly B. Riley FBR, Inc.), Cantor Fitzgerald & Co., National Securities Corporation, and Oppenheimer & Co. Inc. (each an "Agent" and collectively, the "Agents"), relating to the sale of shares of common stock. Under the ATM Agreement, the Company pays the Agents a commission rate of up to 3.0% of the gross proceeds from the sale of any shares of common stock. On December 31, 2020, the ATM Agreement was amended to add H.C. Wainwright & Co., LLC as an Agent.

During the year ended December 31, 2021, the Company issued approximately 19.4 million shares of common stock at an average price of $3.70 per share for gross proceeds of $71.9 million under the ATM Agreement. In connection with these sales, the Company paid aggregate fees of approximately $1.3 million for net proceeds of approximately $70.6 million.

During the year ended December 31, 2020, the Company issued approximately 17.6 million shares of common stock at an average price of $3.40 per share for gross proceeds of $59.8 million under the ATM Agreement. In connection with these sales, the Company paid aggregate fees of approximately $1.1 million for net proceeds of approximately $58.7 million.

Public Offering of Common Stock

On June 11, 2020, we entered into an underwriting agreement (the "Underwriting Agreement") with Cantor Fitzgerald & Co., as representative of the underwriters named therein (each, an "Underwriter" and collectively with Cantor Fitzgerald & Co., the "Underwriters"). In connection with the Underwriting Agreement, we issued 10,769,231 shares of common stock (plus a 30-day option to purchase up to an additional 1,615,384 shares of common stock, of which 686,373 were exercised) at a price of $3.25 per share for gross proceeds of approximately $37.2 million, before deducting underwriting discounts and commissions and offering expenses. In connection with the public offering, we paid aggregate fees of approximately $2.4 million for net proceeds of approximately $34.8 million. The shares were sold under our S-3 registrations filed with the Securities and Exchange Commission. The offering closed on June 15, 2020, and the over-allotment closed on June 25, 2020.

Registration Statements

On April 23, 2021, the Company filed a shelf registration statement No. 333-255476 on Form S-3 (the "2021 S-3"), which was declared effective on May 24, 2021. Under the 2021 S-3, the Company may sell up to a total of $200.0 million of its securities. As of December 31, 2021, there have been no sales of securities under the 2021 S-3.

On October 23, 2020, the Company filed a shelf registration statement No. 333-249657 on Form S-3 (the "2020 S-3"), which was declared effective on December 4, 2020. Under the 2020 S-3, the Company may sell up to a total of $100.0 million of its securities. As of December 31, 2021, approximately $14.6 million of the 2020 S-3 remains available for sales of securities.

Stock Issuances to Fortress

Under the terms of the Second Amended and Restated Founders Agreement, which became effective July 22, 2016, Fortress will receive a grant of shares of our common stock equal to two and one-half percent (2.5%) of the gross amount of any equity or debt financing.

For the year ended December 31, 2021, the Company issued 576,157 shares of common stock and recorded 51,295 shares issuable to Fortress, which equaled 2.5% of the gross proceeds of $71.9 million from the sale of shares of common stock under Mustang's At-the-Market Offering.

For the year ended December 31, 2020, the Company issued 342,773 shares of common stock and recorded 101,632 shares issuable to Fortress, which equaled 2.5% of the gross proceeds of $59.8 million from the sale of shares of common stock under Mustang's At-the-Market Offering.

For the year ended December 31, 2020, the Company issued 286,390 shares of common stock to Fortress, which equaled 2.5% of the gross proceeds of $37.2 million from the sale of shares of common stock, before deducting underwriting discounts and commissions and offering expenses under Mustang's Public Offering.

Equity Incentive Plan

The Company has in effect the 2016 Incentive Plan (the "Incentive Plan"). The Incentive Plan was adopted in 2016 by our stockholders and the compensation committee of the Company's board of directors and is authorized to grant stock-based awards to directors, officers, employees and consultants. The plan initially authorized grants to issue up to 2,000,000 shares of authorized but unissued common stock and expires 10 years from adoption and limits the term of each option to no more than 10 years from the date of grant.

In June 2018, the Company's stockholders approved an amendment to the Incentive Plan to increase the number of authorized shares issuable by 3,000,000 shares, for a total of 5,000,000 shares. In June 2021, the Company's stockholders approved an amendment to the Incentive Plan to increase the number of authorized shares issuable by 3,000,000 shares, for a total of 8,000,000 shares. As of December 31, 2021, 2,823,838 shares are available for issuance of stock-based awards under the Incentive Plan.

Stock Options

The following table summarizes stock option activities for the year ended December 31, 2021 and 2020:

	Stock Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (in years)
Outstanding at December 31, 2019	1,241,675	$ 5.73	7.31
Options forfeited	(100,000)	5.73	—
Outstanding at December 31, 2020	1,141,675	$ 5.73	6.31
Outstanding at December 31, 2021	1,141,675	5.73	5.31
Options vested and exercisable at December 31, 2021	713,547	$ 5.73	5.31

As of December 31, 2021, the Company had no unrecognized stock-based compensation expense related to options. The Company accounts for forfeited awards as they occur as permitted.

Restricted Stock Awards

Certain employees and directors have been awarded restricted stock. The restricted stock vesting consists of milestone and time-based vesting. The following table summarizes restricted stock award activities for the year ended December 31, 2021 and 2020:

	Number of Shares	Weighted Average Grant Date Fair Value
Nonvested at December 31, 2019	299,060	$ 5.66
Granted	83,055	3.08
Vested	(80,001)	5.73
Nonvested at December 31, 2020	302,114	$ 4.93
Granted	68,870	3.63
Vested	(90,001)	6.69
Nonvested at December 31, 2021	280,983	$ 4.05

As of December 31, 2021, the Company had unrecognized stock-based compensation expense related to restricted stock of $0.4 million, which is expected to be recognized over a weighted average period of approximately 1.8 years.

Restricted Stock Units

The following table summarizes restricted stock units' activities for the year ended December 31, 2021 and 2020:

	Number of Units	Weighted Average Grant Date Fair Value
Nonvested at December 31, 2019	1,112,417	$ 5.11
Granted	973,000	2.95
Forfeited	(205,125)	4.24
Vested	(411,733)	4.87
Nonvested at December 31, 2020	1,468,559	$ 3.87
Granted	1,660,250	3.07
Forfeited	(372,873)	3.60
Vested	(420,379)	4.27
Nonvested at December 31, 2021	2,335,557	$ 3.27

As of December 31, 2021, the Company had unrecognized stock-based compensation expense related to restricted stock units of approximately $4.1 million, which is expected to be recognized over a weighted average period of approximately 2.0 years.

The following table summarizes stock-based compensation expense for the years ended December 31, 2021 and 2020 (in thousands).

	For the year ended December 31,	
	2021	2020
General and administrative	$ 1,030	$ 1,550
Research and development	2,278	1,437
Total stock-based compensation expense	$ 3,308	$ 2,987

Stock Warrants

In connection with the Company's offering of shares of common stock in a private placement, each investor received a warrant equal to 25% of the common shares purchased in connection with the offering. Further, National Securities Corporation received Placement Agent Warrants.

A summary of warrant activities for years ended December 31, 2021 and 2020, is presented below:

	Warrants	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (in years)
Outstanding as of December 31, 2019	5,405,669	$ 8.20	2.40
Cashless exercised	(2,999)	—	—
Outstanding as of December 31, 2020	5,402,670	$ 8.21	1.39
Expired	(2,093,878)	8.50	—
Cashless exercised	(138)	—	—
Outstanding as of December 31, 2021	3,308,654	$ 8.02	0.73

Upon the exercise of warrants, the Company will issue new shares of Common Stock.

Employee Stock Purchase Plan

In connection with our Employee Stock Purchase Plan ("ESPP"), eligible employees of Mustang and Fortress can purchase the Company's Common Stock at the end of a predetermined offering period at 85% of the lower of the fair market value at the beginning or end of the offering period.

As of December 31, 2021, 255,177 shares have been purchased and 744,823 shares are available for future sale under the Company's ESPP.

Note 10 - Income Taxes

The Company has accumulated net losses since inception and has not recorded an income tax provision or benefit during the years ended December 31, 2021 and 2020.

A reconciliation of the statutory U.S. federal rate to the Company's effective tax rate is as follows:

	For the year ended December 31,	
	2021	2020
Statutory federal income tax rate	21 %	21 %
State taxes, net of federal tax benefit	16 %	16 %
Non-deductible items	(1)%	(1)%
Credits	5 %	4 %
Federal tax rate change	— %	— %
State tax rate change	— %	1 %
Other	— %	— %
Change in valuation allowance	(41)%	(41)%
Income taxes provision (benefit)	—	—

The components of the net deferred tax asset as of December 31, 2021 and 2020 are the following ($ in thousands):

	For the year ended December 31,	
	2021	2020
Deferred tax assets:		
Net operating loss carryovers	$ 66,879	$ 45,090
Stock compensation and other	2,702	2,444
Change in fair value of warrant liabilities	59	59
Amortization of license	13,977	12,804
Lease liability	755	825
Accruals and reserves	1,035	504
Startup costs	6	7
Tax credits	9,728	5,743
Total deferred tax assets	95,141	67,476
Less: valuation allowance	(94,751)	(67,073)
Net deferred tax assets	$ 390	$ 403
Deferred tax liabilities:		
Right of use asset	(390)	(403)
Total deferred tax assets, net	$ —	$ —

The Company has determined, based upon available evidence, that it is more likely than not that the net deferred tax asset will not be realized and, accordingly, has provided a full valuation allowance against its net deferred tax assets as of December 31, 2021 and 2020. A valuation allowance of approximately $94.8 million and $67.1 million, respectively, was recorded for the years ended December 31, 2021 and 2020.

As of December 31, 2021, the Company had federal and state net operating loss carryforwards of approximately $191.7 million and $410.0 million, respectively. Approximately $168.1 million and $94,000 of the federal and state net operating loss carryforwards, respectively, can be carried forward indefinitely. As of December 31, 2021, the Company had federal and state income tax credits of approximately $8.3 million and $1.8 million, respectively, which will begin to expire in 2033. Under the provisions of Section 382 of the Internal Revenue Code, a corporation that undergoes an "ownership change", as defined therein, is subject to limitations on its use of pre-change NOLs and income tax credits carryforwards

to offset future tax liabilities. Certain tax attributes may be subject to an annual limitation as a result of the Company's January 2017 capital raise, as it appears to constitute an ownership change under Section 382. The Company has recorded a full valuation allowance on all of its deferred tax assets as it believes that it is more likely than not that the deferred tax assets will not be realized regardless of whether an "ownership change" has occurred.

There are no significant items determined to be unrecognized tax benefits taken or expected to be taken in a tax return, in accordance with ASC 740 "Income Taxes" ("ASC 740"), which clarifies the accounting for uncertainty in income taxes recognized in the financial statements, that have been recorded on the Company's financial statements for the periods ended December 31, 2021 and 2020. The Company does not anticipate a material change to unrecognized tax benefits in the next twelve months.

Additionally, ASC 740 provides guidance on the recognition of interest and penalties related to income taxes. There were no interest or penalties related to income taxes that have been accrued or recognized as of and for the periods ended December 31, 2021 and 2020.

The company is subject to U.S. federal and various state taxes. As of December 31, 2021, the earliest federal tax year open for the assessment of income taxes under the applicable statutes of limitations is its 2018 tax year.

In response to the COVID-19 pandemic, the Coronavirus Aid, Relief and Economic Security Act ("CARES Act") was signed into law on March 27, 2020. The CARES Act, among other things, includes tax provisions relating to refundable payroll tax credits, deferment of employer's social security payments, net operating loss utilization and carryback periods and modifications to the net interest deduction limitations. The CARES Act did not have a material impact on the Company's income tax provision for 2021. The Company will continue to evaluate the impact of the CARES Act on its financial position, results of operations and cash flows.

On December 27, 2020, the President of the United States signed the Consolidated Appropriations Act, 2021 ("Consolidated Appropriations Act") into law. The Consolidated Appropriations Act is intended to enhance and expand certain provisions of the CARES Act, allows for the deductions of expenses related to the Payroll Protection Program funds received by companies, and provides an update to meals and entertainment expensing for 2021. The Consolidated Appropriations Act did not have a material impact to the Company's income tax provision for 2021.

Note 11 – Subsequent Events

On March 8, 2022, the Company announced completion of a $75 million long-term debt facility with Runway Growth Capital LLC ("Runway"). Of the $75 million, $30 million was funded upon closing, and the additional $45 million available under the facility may be funded upon Mustang's achieving certain predetermined milestones. The loan will be repaid in sixty monthly payments consisting of 24 monthly payments of interest only, followed by 36 monthly payments of principal and accrued interest, payable monthly in arrears, with all repayments ending on the same date as the initial tranche. The interest-only period may be extended to 36 months contingent upon Mustang achieving certain milestones.

In connection with the debt financing, Mustang issued to Runway warrants to purchase up to 748,036 of its common shares at an exercise price of $0.8021 per share. Proceeds from the facility will be used to support the ongoing clinical development of key investigational product candidates within Mustang's pipeline and for general working capital purposes.

SIGNATURES

Pursuant to the requirements of Section 12 of the Securities Exchange Act of 1934, the registrant has duly caused this Form 10-K to be signed on its behalf by the undersigned, thereunto duly authorized.

Mustang Bio, Inc.

By: /s/ Manuel Litchman
Name: Manuel Litchman
Title: President and Chief Executive Officer

March 23, 2022

POWER OF ATTORNEY

We, the undersigned directors and/or executive officers of Mustang Bio, Inc., hereby severally constitute and appoint Manuel Litchman, acting singly, his or her true and lawful attorney-in-fact and agent, with full power of substitution and resubstitution, for him or her in any and all capacities, to sign this Form 10-K and to file the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorney-in-fact and agent full power and authority to do and perform each and every act and thing necessary or appropriate to be done in connection therewith, as fully for all intents and purposes as he or she might or could do in person, hereby approving, ratifying and confirming all that said attorney-in-fact and agent, or his substitute, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this Form 10-K has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Michael S. Weiss Michael S. Weiss	Executive Chairman of the Board	March 23, 2022
/s/ Manuel Litchman Manuel Litchman, M.D.	President and Chief Executive Officer	March 23, 2022
/s/ Lindsay A. Rosenwald Lindsay A. Rosenwald, M.D.	Director	March 23, 2022
/s/ Neil Herskowitz Neil Herskowitz	Director	March 23, 2022
/s/ Adam Chill Adam Chill	Director	March 23, 2022
/s/ Michael Zelefsky Michael Zelefsky, M.D.	Director	March 23, 2022
/s/ Brian Achenbach Brian Achenbach	Senior Vice President of Finance & Corporate Controller	March 23, 2022

EXHIBIT 4.3

DESCRIPTION OF THE REGISTRANT'S SECURITIES REGISTERED PURSUANT TO SECTION 12 OF THE SECURITIES EXCHANGE ACT OF 1934

DESCRIPTION OF CAPITAL STOCK

When used herein, the terms "Company," "we," "our," and "us" refer to Mustang Bio, Inc.

Capital Stock

The Company is authorized to issue 125,000,000 shares of common stock with a par value of $0.0001 per share, of which 1,000,000 shares are designated as Class A common stock and 2,000,000 of preferred stock at $0.0001 par value of which 250,000 are designated as Class A preferred stock.

The holders of common stock are entitled to one vote per share of common stock held.

The undesignated preferred stock may be issued from time to time in one or more series. The Board of Directors is authorized to determine or alter the dividend rights, dividend rate, conversion rights, voting rights, rights and terms of redemption (including sinking fund provisions, if any), the redemption price or prices, the liquidation preferences and other designations, powers, preferences and relative, participating, optional or other special rights, if any, and the qualifications, limitations and restrictions granted to or imposed upon any wholly unissued series of preferred stock, and to fix the number of shares of any series of preferred stock (but not below the number of shares of any such series then outstanding).

Class A Common Stock

The holders of Class A common stock are entitled to the number of votes equal to the number of whole shares of common stock into which the shares of Class A common shares held by such holder are convertible. For a period of ten years from issuance, the holders of the Class A common stock have the right to appoint one member of the board of directors of Mustang. To date, the holders of Class A common stock have not yet appointed such director.

Class A Preferred Stock

The Class A Preferred Stock is identical to undesignated common stock other than as to voting rights, conversion rights, and the PIK dividend right.

The holders of the outstanding shares of Class A Preferred Stock receive on each January 1 (each a "PIK Dividend Payment Date") after the original issuance date of the Class A Preferred Stock until the date all outstanding Class A Preferred Stock is converted into common stock or redeemed (and the purchase price is paid in full), pro rata per share dividends paid in additional fully paid and non-assessable shares of common stock such that the aggregate number of shares of common stock issued pursuant to such PIK dividend is equal to 2.5% of the Corporation's fully-diluted outstanding capitalization on the date that is one business day prior to any PIK Dividend Payment Date ("PIK Record Date"). In the event the Class A Preferred Stock converts into common stock, the holders shall receive all PIK dividends accrued through the date of such conversion. No dividend or other distribution shall be paid, or declared and set apart for payment (other than dividends payable solely in capital stock on the capital stock) on the shares of common stock until all PIK dividends on the Class A Preferred Stock shall have been paid or declared and set apart for payment. All dividends are non-cumulative.

On any matter presented to the stockholders for their action or consideration at any meeting of stockholders (or by written consent of stockholders in lieu of meeting), each holder of outstanding shares of Class A Preferred Stock shall be entitled to cast for each share of Class A Preferred Stock held by such holder as of the record date for determining stockholders entitled to vote on such matter, the number of votes that is equal to one and one-tenth (1.1) times a fraction, the numerator of which is the sum of (A) the number of shares of outstanding common stock and (B) the whole shares of common stock in to which the shares of outstanding Class A Common Stock and the Class A Preferred Stock are convertible,

and the denominator of which is number of shares of outstanding Class A Preferred Stock. Thus, the Class A Preferred Stock will at all times constitute a voting majority.

Each share of Class A Preferred Stock is convertible, at the option of the holder, into one fully paid and nonassessable share of common stock, subject to certain adjustments. If the Company, at any time effects a subdivision or combination of the outstanding common stock (by any stock split, stock dividend, recapitalization, reverse stock split or otherwise), the applicable conversion ratio in effect immediately before that subdivision is proportionately decreased or increased, as applicable, so that the number of shares of common stock issuable on conversion of each share of Class A Preferred Stock shall be increased or decreased, as applicable, in proportion to such increase or decrease in the aggregate number of shares of common stock outstanding. Additionally, if any reorganization, recapitalization, reclassification, consolidation or merger involving the Company occurs in which the common stock (but not the Class A Preferred Stock) is converted into or exchanged for securities, cash or other property, then each share of Class A Preferred Stock becomes convertible into the kind and amount of securities, cash or other property which a holder of the number of shares of common stock of the Company issuable upon conversion of one share of the Class A Preferred Stock immediately prior to such reorganization, recapitalization, reclassification, consolidation or merger would have been entitled to receive pursuant to such transaction.

Additional Features

Other features of our capital stock include:

- *Dividend Rights*. The holders of outstanding shares of our common stock, including Class A common stock, are entitled to receive dividends out of funds legally available at the times and in the amounts that our board of directors may determine. All dividends are non-cumulative.

- *Voting Rights*. The holders of our common stock are entitled to one vote for each share of common stock held on all matters submitted to a vote of the stockholders, including the election of directors. Our certificate of incorporation and bylaws do not provide for cumulative voting rights.

- *No Preemptive or Similar Rights*. The holders of our common stock have no preemptive, conversion, or subscription rights, and there are no redemption or sinking fund provisions applicable to our common stock.

- *Right to Receive Liquidation Distributions*. Upon our liquidation, dissolution, or winding-up, the assets legally available for distribution to our stockholders would be distributable ratably among the holders of our common stock, including Class A common stock, outstanding at that time after payment of other claims of creditors, if any.

- *Fully Paid and Non-Assessable*. All of the outstanding shares of our common stock, including Class A common stock, and the Class A Preferred Stock are duly issued, fully paid and non-assessable.

DESCRIPTION OF WARRANTS

The terms relating to any warrants to purchase shares of our common stock or preferred stock, in one or more series together with other securities or separately, will include some or all of the following:

- the title of the warrants;

- the aggregate number of warrants offered;

- the designation, number and terms of the shares of common stock purchasable upon exercise of the warrants and procedures by which those numbers may be adjusted;

- the exercise price of the warrants;

- the dates or periods during which the warrants are exercisable;
- the designation and terms of any securities with which the warrants are issued;
- if the warrants are issued as a unit with another security, the date on and after which the warrants and the other security will be separately transferable;
- if the exercise price is not payable in U.S. dollars, the foreign currency, currency unit or composite currency in which the exercise price is denominated;
- any minimum or maximum amount of warrants that may be exercised at any one time;
- any terms relating to the modification of the warrants;
- any terms, procedures and limitations relating to the transferability, exchange or exercise of the warrants; and
- any other specific terms of the warrants.

DESCRIPTION OF DEBT SECURITIES

We may offer debt securities which may be senior, subordinated or junior subordinated and may be convertible. Unless otherwise specified, our debt securities will be issued in one or more series under an indenture to be entered into between us and a trustee. The debt securities will be issued under an indenture to be entered into between us and a trustee. The terms of the debt securities will include those stated in the indenture and those made part of the indenture by reference to the Trust Indenture Act of 1939, as in effect on the date of the indenture. The indenture will be subject to and governed by the terms of the Trust Indenture Act of 1939.

The following description briefly sets forth certain general terms and provisions of the debt securities that we may offer.

Debt Securities

The aggregate principal amount of debt securities that may be issued under the indenture is unlimited. The debt securities may be issued in one or more series as may be authorized from time to time pursuant to a supplemental indenture entered into between us and the trustee or an order delivered by us to the trustee. Debt securities we offer will be subject to the following terms and conditions of the series of debt securities being offered, to the extent applicable:

- title and aggregate principal amount;
- whether the debt securities will be senior, subordinated or junior subordinated;
- applicable subordination provisions, if any;
- provisions regarding whether the debt securities will be convertible or exchangeable into other securities or property of the Company or any other person;
- percentage or percentages of principal amount at which the debt securities will be issued;
- maturity date(s);
- interest rate(s) or the method for determining the interest rate(s);
- whether interest on the debt securities will be payable in cash or additional debt securities of the same series;
- dates on which interest will accrue or the method for determining dates on which interest will accrue and dates on which interest will be payable;
- whether the amount of payment of principal of, premium, if any, or interest on the debt securities may be determined with reference to an index, formula or other method;
- redemption, repurchase or early repayment provisions, including our obligation or right to redeem, purchase or repay debt securities under a sinking fund, amortization or analogous provision;
- if other than the debt securities' principal amount, the portion of the principal amount of the debt securities that will be payable upon declaration of acceleration of the maturity;
- authorized denominations;
- form;

- amount of discount or premium, if any, with which the debt securities will be issued, including whether the debt securities will be issued as "original issue discount" securities;
- the place or places where the principal of, premium, if any, and interest on the debt securities will be payable;
- where the debt securities may be presented for registration of transfer, exchange or conversion;
- the place or places where notices and demands to or upon the Company in respect of the debt securities may be made;
- whether the debt securities will be issued in whole or in part in the form of one or more global securities;
- if the debt securities will be issued in whole or in part in the form of a book-entry security, the depository or its nominee with respect to the debt securities and the circumstances under which the book-entry security may be registered for transfer or exchange or authenticated and delivered in the name of a person other than the depository or its nominee;
- whether a temporary security is to be issued with respect to such series and whether any interest payable prior to the issuance of definitive securities of the series will be credited to the account of the persons entitled thereto;
- the terms upon which beneficial interests in a temporary global security may be exchanged in whole or in part for beneficial interests in a definitive global security or for individual definitive securities;
- the guarantors, if any, of the debt securities, and the extent of the guarantees and any additions or changes to permit or facilitate guarantees of such debt securities;
- any covenants applicable to the particular debt securities being issued;
- any defaults and events of default applicable to the debt securities, including the remedies available in connection therewith;
- currency, currencies or currency units in which the purchase price for, the principal of and any premium and any interest on, such debt securities will be payable;
- time period within which, the manner in which and the terms and conditions upon which the Company or the purchaser of the debt securities can select the payment currency;
- securities exchange(s) on which the debt securities will be listed, if any;
- whether any underwriter(s) will act as market maker(s) for the debt securities;
- extent to which a secondary market for the debt securities is expected to develop;
- provisions relating to defeasance;
- provisions relating to satisfaction and discharge of the indenture;
- any restrictions or conditions on the transferability of the debt securities;
- provisions relating to the modification of the indenture both with and without the consent of holders of debt securities issued under the indenture;

- any addition or change in the provisions related to compensation and reimbursement of the trustee;
- provisions, if any, granting special rights to holders upon the occurrence of specified events;
- whether the debt securities will be secured or unsecured, and, if secured, the terms upon which the debt securities will be secured and any other additions or changes relating to such security; and
- any other terms of the debt securities that are not inconsistent with the provisions of the Trust Indenture Act (but may modify, amend, supplement or delete any of the terms of the indenture with respect to such series of debt securities).

General

One or more series of debt securities may be sold as "original issue discount" securities. These debt securities would be sold at a substantial discount below their stated principal amount, bearing no interest or interest at a rate which at the time of issuance is below market rates. One or more series of debt securities may be variable rate debt securities that may be exchanged for fixed rate debt securities.

Debt securities may be issued where the amount of principal and/or interest payable is determined by reference to one or more currency exchange rates, commodity prices, equity indices or other factors. Holders of such debt securities may receive a principal amount or a payment of interest that is greater than or less than the amount of principal or interest otherwise payable on such dates, depending upon the value of the applicable currencies, commodities, equity indices or other factors.

The term "debt securities" includes debt securities denominated in U.S. dollars or, if applicable, in any other freely transferable currency or units based on or relating to foreign currencies.

We expect most debt securities to be issued in fully registered form without coupons and in denominations of $1,000 and any integral multiples thereof. Subject to applicable limitations, debt securities that are issued in registered form may be transferred or exchanged at the principal corporate trust office of the trustee, without the payment of any service charge, other than any tax or other governmental charge payable in connection therewith.

Global Securities

The debt securities of a series may be issued in whole or in part in the form of one or more global securities that will be deposited with, or on behalf of, a depositary. Global securities will be issued in registered form and in either temporary or definitive form. Unless and until it is exchanged in whole or in part for the individual debt securities, a global security may not be transferred except as a whole by the depositary for such global security to a nominee of such depositary or by a nominee of such depositary to such depositary or another nominee of such depositary or by such depositary or any such nominee to a successor of such depositary or a nominee of such successor.

Governing Law

The indenture and the debt securities shall be construed in accordance with and governed by the laws of the State of New York.

DESCRIPTION OF UNITS

We may issue, in one more series, units comprised of shares of our common stock, preferred stock, warrants to purchase common stock or preferred stock, debt securities or any combination of those securities. Each unit will be issued so that the holder of the unit is also the holder of each security included in the unit. Thus, the holder of a unit will have the rights and obligations of a holder of each included security. The unit agreement under which a unit is issued may provide that the securities included in the unit may not be held or transferred separately, at any time or at any time before a specified date.

We may evidence units by unit certificates that we issue under a separate agreement. We may issue the units under a unit agreement between us and one or more unit agents. If we elect to enter into a unit agreement with a unit agent, the unit agent will act solely as our agent in connection with the units and will not assume any obligation or relationship of agency or trust for or with any registered holders of units or beneficial owners of units.

The terms of the series of units that may be offered will include:

- the designation and terms of the units and of the securities comprising the units, including whether and under what circumstances those securities may be held or transferred separately;
- any provisions of the governing unit agreement that differ from those described herein; and
- any provisions for the issuance, payment, settlement, transfer or exchange of the units or of the securities comprising the units.

The other provisions regarding our common stock, preferred stock, warrants and debt securities as described in this exhibit will apply to each unit to the extent such unit consists of shares of our common stock, warrants and/or debt securities.

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

Mustang Bio, Inc.
New York, New York

We hereby consent to the incorporation by reference in the Registration Statements on Form S-3 (Nos. 333-255476, 333-226175, 333-233350 and 333-249657) and Form S-8 (Nos. 333-258310, 333-258311, 333-255007, 333-221819) of Mustang Bio, Inc. of our report dated March 24, 2021 relating to the financial statements, which appears in this Annual Report on Form 10-K.

/s/ BDO USA, LLP
Boston, Massachusetts
March 23, 2022

Exhibit 23.2

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the registration statements (Nos. 333-255476, 333-226175, 333-233350, and 333-249657) on Form S-3 and in the registration statements (Nos. 333-258310, 333-258311, 333-225007, and 333-221819) on Form S-8 of our report dated March 23, 2022, with respect to the financial statements of Mustang Bio Inc.

/s/ KPMG LLP

Hartford, CT
March 23, 2022

EXHIBIT 31.1

CERTIFICATION PURSUANT TO

SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Manuel Litchman, M.D., President and Chief Executive Officer (Principal Executive Officer), certify that:

(1) I have reviewed this Annual Report on Form 10-K for the year ended December 31, 2021 of Mustang Bio, Inc. (the registrant);

(2) Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

(3) Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects, the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

(4) The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) disclosed in the report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

(5) The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: March 23, 2022

By: /s/ Manuel Litchman
Manuel Litchman, M.D.
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION PURSUANT TO

SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Brian Achenbach, Senior Vice President of Finance & Corporate Controller (Principal Financial Officer), certify that:

(1) I have reviewed this Annual Report on Form 10-K for the year ended December 31, 2021 of Mustang Bio, Inc. (the registrant);

(2) Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

(3) Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects, the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

(4) The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) disclosed in the report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

(5) The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: March 23, 2022

By: /s/ Brian Achenbach
Brian Achenbach
Senior Vice President of Finance & Corporate Controller
(Principal Financial Officer)

EXHIBIT 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Mustang Bio, Inc. (the "Company") for the period ended December 31, 2021, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Manuel Litchman, M.D., President and Chief Executive Officer, hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company as of, and for, the periods presented in the Report.

Dated: March 23, 2022

By: /s/ Manuel Litchman
Manuel Litchman, M.D.,
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Mustang Bio, Inc. (the "Company") for the period ended December 31, 2021, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Brian Achenbach, Senior Vice President Finance & Corporate Controller, hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company, as of, and for, the periods presented in the Report.

Dated: March 23, 2022

By: /s/ Brian Achenbach
Brian Achenbach
Senior Vice President of Finance & Corporate Controller
(Principal Financial Officer)