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

FORM 10-Q

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

For the quarterly period ended March 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 Number 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, MA 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

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 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 May 13, 2021**
Class A Common Stock, $0.0001 par value	845,385
Common Stock, $0.0001 par value	86,467,920

MUSTANG BIO, INC.
QUARTERLY REPORT ON FORM 10-Q
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SUMMARY OF 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. FINANCIAL INFORMATION
Item 1. Financial Statements

MUSTANG BIO, INC.
Condensed Balance Sheets
(in thousands, except share and per share amounts)

	March 31, 2021 (Unaudited)	December 31, 2020
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 129,371	$ 97,804
Other receivables - related party	17	15
Prepaid expenses and other current assets	1,581	1,715
Total current assets	130,969	99,534
Property, plant and equipment, net	7,080	7,529
Fixed assets - construction in process	1,458	499
Restricted cash	1,000	1,000
Other assets	255	250
Operating lease right-of-use asset, net	1,060	1,088
Total Assets	**$ 141,822**	**$ 109,900**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current Liabilities:		
Accounts payable and accrued expenses	$ 5,959	$ 8,747
Payables and accrued expenses - related party	343	490
Operating lease liabilities - short-term	284	278
Total current liabilities	**6,586**	**9,515**
Operating lease liabilities - long-term	1,875	1,950
Total Liabilities	**8,461**	**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 March 31, 2021 and December 31, 2020, respectively	—	—
Common Stock ($0.0001 par value), 125,000,000 shares authorized		
Class A common shares, 845,385 shares issued and outstanding as of March 31, 2021 and December 31, 2020, respectively	—	—
Common shares, 85,043,153 and 70,920,693 shares issued and outstanding as of March 31, 2021 and December 31, 2020, respectively	8	7
Common stock issuable, 63,688 and 2,103,122 shares as of March 31, 2021 and December 31, 2020, respectively	218	7,939
Additional paid-in capital	333,566	275,963
Accumulated deficit	(200,431)	(185,474)
Total Stockholders' Equity	**133,361**	**98,435**
Total Liabilities and Stockholders' Equity	**$ 141,822**	**$ 109,900**

The accompanying notes are an integral part of these condensed financial statements.

MUSTANG BIO, INC.
Condensed Statements of Operations
(in thousands, except share and per share amounts)
(Unaudited)

	For the three months ended March 31,	
	2021	**2020**
Operating expenses:		
Research and development	$ 11,618	$ 9,314
Research and development – licenses acquired	—	250
General and administrative	3,469	1,956
Total operating expenses	15,087	11,520
Loss from operations	(15,087)	(11,520)
Other income (expense)		
Interest income	134	263
Interest expense	(4)	(600)
Total other income (expense)	130	(337)
Net Loss	**$ (14,957)**	**$ (11,857)**
Net loss per common share outstanding, basic and diluted	$ (0.19)	$ (0.28)
Weighted average number of common shares outstanding, basic and diluted	80,466,049	41,971,316

The accompanying notes are an integral part of these condensed financial statements.

MUSTANG BIO, INC.
Condensed Statements of Stockholders' Equity
(in thousands, except share amounts)
(Unaudited)

	For the Three Months Ended March 31, 2021									
	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, 2020	**250,000**	**$ —**	**845,385**	**$ —**	**70,920,693**	**$ 7**	**$ 7,939**	**$ 275,963**	**$ (185,474)**	**$ 98,435**
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	—	—	—	—	11,597,503	1	—	47,530	—	47,531
Issuance of common shares, equity fee on At-the-Market Offering	—	—	—	—	325,221	—	(144)	1,342	—	1,198
Issuance of common shares under ESPP	—	—	—	—	54,920	—	—	158	—	158
Stock-based compensation expenses	—	—	—	—	143,188	—	—	996	—	996
Exercise of warrants	—	—	—	—	138	—	—	—	—	—
Net loss	—	—	—	—	—	—	—	—	(14,957)	(14,957)
Balances at March 31, 2021	**250,000**	**$ —**	**845,385**	**$ —**	**85,043,153**	**$ 8**	**$ 218**	**$ 333,566**	**$ (200,431)**	**$ 133,361**

	For the Three Months Ended March 31, 2020									
	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**
Issuance of common shares - Founders Agreement	—	—	—	—	1,206,667	—	(4,923)	4,923	—	—
Issuance of common shares, net of offering shares -At-the-Market Offering	—	—	—	—	1,248,834	—	—	4,910	—	4,910
Issuance of common shares - Equity fee on At-the-Market Offering	—	—	—	—	31,220	—	—	125	—	125
Issuance of common shares under ESPP	—	—	—	—	68,351	—	—	169	—	169
Stock-based compensation expenses	—	—	—	—	115,250	—	—	805	—	805
Exercise of warrants	—	—	—	—	2,999	—	—	—	—	—
Net loss	—	—	—	—	—	—	—	—	(11,857)	(11,857)
Balances at March 31, 2020	**250,000**	**$ —**	**845,385**	**$ —**	**42,076,840**	**$ 4**	**$ —**	**$ 183,116**	**$ (137,316)**	**$ 45,804**

The accompanying notes are an integral part of these condensed financial statements.

MUSTANG BIO, INC.
Condensed Statements of Cash Flows
(in thousands)
(Unaudited)

	For the three months ended March 31,	
	2021	**2020**
Cash Flows from Operating Activities:		
Net loss	$ (14,957)	$ (11,857)
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	1,198	125
Research and development - licenses acquired	—	250
Stock-based compensation expenses	996	805
Depreciation expense	462	373
Accretion of debt discount	—	259
Amortization of operating lease right-of-use assets	28	31
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	129	(246)
Other receivables - related party	(2)	5
Accounts payable and accrued expenses	(3,218)	(163)
Payable and accrued expenses - related party	(147)	(50)
Lease liabilities	(69)	316
Net cash used in operating activities	(15,580)	(10,152)
Cash Flows from Investing Activities:		
Purchase of fixed assets	(458)	(526)
Net cash used in investing activities	(458)	(526)
Cash Flows from Financing Activities:		
Proceeds from issuance of common shares - At-the-Market Offering	48,379	4,997
Offering costs for the issuance of common shares - At-the-Market Offering	(932)	(87)
Proceeds from issuance of common shares under ESPP	158	169
Net cash provided by financing activities	47,605	5,079
Net change in cash, cash equivalents and restricted cash	31,567	(5,599)
Cash, cash equivalents and restricted cash, beginning of the period	98,804	62,413
Cash, cash equivalents and restricted cash, end of the period	**$ 130,371**	**$ 56,814**
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ —	$ 341
Supplemental disclosure of noncash investing and financing activities:		
Fixed assets (acquired but not paid)	$ 545	$ 540
Issuance of common shares - Founders Agreement	$ 7,577	$ 4,923
Research and development licenses included in accounts payable and accrued expenses	$ —	$ 250

The accompanying notes are an integral part of these condensed financial statements

Note 1 - Organization, Description of Business and Liquidity and Capital Resources

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").

The Company's common stock is listed on the NASDAQ Global Market and trades under the symbol "MBIO".

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 March 31, 2021, the Company had an accumulated deficit of $200.4 million.

The Company has funded its operations to date primarily through the sale of equity. 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 March 31, 2021, are sufficient to fund its anticipated operating cash requirements for at least one year from the filing date of this Form 10-Q.

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 spread of COVID-19 and the actions implemented to combat the virus throughout the world.

Note 2 - Significant Accounting Policies

Basis of Presentation

The accompanying unaudited interim condensed financial statements have been prepared in accordance with generally accepted accounting principles in the United States of America ("GAAP") for interim financial information and the instructions to Form 10-Q and Article 10 of Regulation S-X of the Exchange Act. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. In the opinion of management, the unaudited interim condensed financial statements reflect all adjustments, which include only normal recurring adjustments necessary for the fair statement of the balances and results for the periods presented. They may not include all of the information and footnotes required by GAAP for complete financial statements. Therefore, these financial statements should be read in conjunction with the Company's audited financial statements and notes thereto for the year ended December 31, 2020, which were included in the Company's Form 10-K and filed with the SEC on March 24, 2021. The results of operations for any interim periods are not necessarily indicative of the results that may be expected for the entire fiscal year or any other interim period.

Use of Estimates

The Company's unaudited condensed 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, 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. Due to the uncertainty inherent in such estimates actual results could differ from those estimates.

Significant Accounting Policies

There have been no material changes to the Company's significant accounting policies previously disclosed in the Company's Form 10-K filed with the SEC on March 24, 2021.

Recently Issued Accounting Standards

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 three months ended March 31, 2021 and 2020, the Company recorded the following expense in research and development for licenses acquired:

	For the three months ended March 31,	
($ in thousands)	**2021**	**2020**
City of Hope National Medical Center		
HER2	—	250
Total	$ —	$ 250

City of Hope

HER2 License (MB-103)

On May 31, 2017, the Company entered into an exclusive license agreement with City of Hope National Medical Center ("COH") for the use of human epidermal growth factor receptor 2 ("HER2") chimeric antigen receptor ("CAR") engineered T cell ("CAR T") technology, which will initially be applied in the treatment of glioblastoma multiforme and brain metastases from HER2+ malignancies. 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 three months ended, March 31, 2021 and March 31, 2020, the Company recordednil and $0.3 million, respectively, in connection with the HER2 license agreement with COH. The $0.3 million represented a non-refundable milestone payment in connection with the twelfth patient treated in the Phase 1 clinical study of MB-103 at COH, for the three months ended March 31, 2020.

Research and Development Expenses - Sponsored Research and Clinical Trial Agreements

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

	For the three months ended March 31,	
($ in thousands)	**2021**	**2020**
City of Hope National Medical Center	$ —	$ 500
CD123	205	230
IL13Rα2	514	92
CS1	175	—
HER2	123	—
PSCA	50	—
Fred Hutchinson Cancer Research Center - CD20	671	527
St. Jude Children's Research Hospital - XSCID	104	—
Total	$ 1,842	$ 1,349

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 the IL13Rα2-directed CAR T program, the CD123-directed CAR T program and the Spacer technology. For the three months ended March 31, 2021 and 2020, the Company recorded expense of nil and $0.5 million, respectively, in research and development expenses in the Condensed 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 the CD123-directed CAR T program (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 the CD123-directed CAR T program. For the three months ended March 31, 2021 and 2020, the Company recorded $0.2 million and $0.2 million, respectively, in research and development expenses in the Condensed 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 the IL13Rα2-directed CAR T program (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 the IL13Rα2-directed CAR T program.

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 program.

For the three months ended March 31, 2021 and 2020, the Company recorded $0.5 million and $0.1 million, respectively, in research and development expenses in the Condensed Statements of Operations pursuant to the terms of these agreements.

CS1 (MB-104) Clinical Research and Support Agreement with City of Hope

In June 2020, Mustang entered into a clinical research and 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 by the Company as MB-104. Under the terms of the agreement Mustang paid COH $0.8 million for costs incurred and will reimburse COH for costs associated with this trial, when incurred, not to exceed $2.4 million. The agreement will expire upon the delivery of a final study report or earlier. For the three months ended March 31, 2021 and 2020, the Company recorded $0.2 million and nil, respectively, in research and development expenses in the Condensed 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 paid 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 three months ended March 31, 2021 and 2020, the Company recorded $0.1 million and nil, respectively, in research and development expenses in the Condensed 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 paid 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 three months ended March 31, 2021 and 2020, the Company recorded $0.1 million and nil, respectively, in research and development expenses in the Condensed Statements of Operations pursuant to the terms of this agreement.

Fred Hutchinson Cancer Research Center

CD20 (MB-106) Clinical Trial Agreement

On July 3, 2017, in conjunction with the CD20 Technology License from Fred Hutchinson Cancer Research Center ("Fred Hutch"), we 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. 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 $0.8 million for the treatment of five patients with chronic lymphocytic leukemia. For the three months ended March 31, 2021 and 2020, the Company recorded $0.7 million and $0.5 million, respectively, in research and development expenses in the Condensed 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, Mustang entered into a Data Transfer Agreement with St. Jude Children's Research Hospital ("St. Jude") under which Mustang will reimburse St. Jude for costs associated with St. Jude's clinical trial for the treatment of infants with X-linked Severe Combined Immunodeficiency ("XSCID"). Pursuant to the terms of this agreement Mustang paid an upfront fee of $1.1 million on July 1, 2020, and will continue to reimburse St. Jude for costs incurred in connection with this trial. For the three months ended March 31, 2021 and 2020, the Company recorded $0.1 million and nil, respectively, in research and development expenses in the Condensed Statements of Operations pursuant to the terms of this agreement.

Note 4 - Related Party Agreements

Founders Agreement and Management Services Agreement with Fortress

In connection with the Company's Management Services Agreement (the "MSA") with Fortress for the three months ended March 31, 2021 and 2020, respectively, expenses related to the MSA are recorded 50%in research and development expenses and 50% in general and administrative expenses in the Statements of Operations. For the three months ended March 31, 2021 and 2020, respectively, the Company recorded expense of $0.1 million and $0.1 million, respectively, related to this agreement.

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. In March 2021, the Company issued 325,221 shares of common stock to Fortress, which equaled2.5% of the gross proceeds of $48.4 million from the sale of shares of common stock under Mustang's At-the-Market Offering. The Company recorded an expense of approximately $1.2 million in general and administrative expenses related to these shares during the three months ended March 31, 2021.

Annual Stock Dividend

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.

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.

Note 5 - Property and Equipment

At March 31, 2021 and December 31, 2020, Mustang's property and equipment consisted of the following:

($ in thousands)	Estimated Useful Life (in years)	March 31, 2021	December 31, 2020
Computer equipment	3	$ 68	$ 68
Furniture and fixtures	5	194	181
Machinery and equipment	5	5,748	5,748
Leasehold improvements	9	5,099	5,099
Construction in process	N/A	1,458	499
Total property and equipment		12,567	11,595
Less: accumulated depreciation		(4,029)	(3,567)
Property and equipment, net		$ 8,538	$ 8,028

Depreciation expense for the three months ended March 31, 2021, and 2020, was approximately $0.5 million and $0.4 million and was recorded in research and development expense in the Condensed Statements of Operations.

Note 6 - Accounts Payable and Accrued Expenses

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

($ in thousands)	March 31, 2021	December 31, 2020
Accounts payable	$ 2,918	$ 3,518
Research and development	1,968	2,862
Accrued compensation	844	2,009
Other	229	358
Total accounts payable and accrued expenses	$ 5,959	$ 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, we 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 we post an initial security deposit of $0.8 million, in the form of a $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 copier, which expires in 2021. 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 March 31, 2021, the Company had operating lease liabilities of $2.2 million and right of use assets of $1.1 million, which were included in the Condensed Balance Sheet.

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

	For the Three Months Ended	
($ in thousands)	March 31, 2021	March 31, 2020
Lease cost		
Operating lease cost	$ 76	$ 83
Variable lease cost	147	234
Total	$ 223	$ 317

($ in thousands)	For the Three Months Ended March 31, 2021	March 31, 2020
Operating cash flows from operating leases	$ 118	$ (270)
Weighted-average remaining lease term – operating leases	5.6	3.1
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, 2021	$ 349
Year ended December 31, 2022	476
Year ended December 31, 2023	489
Year ended December 31, 2024	503
Year ended December 31, 2025	516
Thereafter	439
Total	2,772
Less present value discount	(613)
Operating lease liabilities	$ 2,159

Note 8 - Stockholders' Equity

Registration Statements

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 March 31, 2021, approximately $37.8 million of the 2020 S-3 remains available for sales of securities.

Common Stock

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 Mustang S-3"), which was declared effective in August 2018. Under the 2018 Mustang S-3, the Company may sell up to a total of $75.0 million of its securities. In connection with the 2018 Mustang S-3, the Company entered into an At-the-Market Issuance Sales Agreement (the "Mustang ATM") 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 Mustang ATM, 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 Mustang ATM was amended to add H.C. Wainwright &Co., LLC as an Agent.

During the three months ended March 31, 2021, the Company issued approximately11.6 million shares of common stock at an average price of $4.17 per share for gross proceeds of $48.4 million under the Mustang ATM. In connection with these sales, we paid aggregate fees of approximately $0.9 million for net proceeds of approximately $47.5 million. During the three months ended March 31, 2020, the Company issued approximately 1.2 million shares of common stock at an average price of $3.93 per share for gross proceeds of $5.0 million under the Mustang ATM. In connection with these sales, we paid aggregate fees of approximately $ 0.1 million for net proceeds of approximately $4.9 million.

Pursuant to the Founders Agreement, we issued325,221 shares of common stock to Fortress at a weighted average price of $4.16 per share for the three months ended March 31, 2021 and recorded 63,688 shares issuable to Fortress in connection with the Mustang ATM offering noted above. During the three months ended March 31, 2020, we issued 31,220 shares of common stock to Fortress at a weighted average price of $4.00 per share in connection with the Mustang ATM.

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, 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.

As of March 31, 2021, 636,585 shares are available for issuance under the Incentive Plan.

Stock Options

The following table summarizes stock option activities for the three months ended March 31, 2021:

	Stock Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (in years)
Outstanding at December 31, 2020	1,141,675	5.73	6.31
Outstanding at March 31, 2021	1,141,675	5.73	6.06
Options vested and exercisable at March 31, 2021	820,579	$ 5.73	6.06

As of March 31, 2021, the Company had unrecognized stock-based compensation expense related to options of $0.1 million, which is expected to be recognized over the remaining weighted average vesting period of approximately 0.6 years.

Restricted Stock

The following table summarizes restricted stock award activities for the three months ended March 31, 2021:

	Number of Shares	Weighted Average Grant Date Fair Value
Nonvested at December 31, 2020	302,114	$ 4.93
Nonvested at March 31, 2021	302,114	$ 4.93

As of March 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 the remaining weighted average vesting period of approximately 1.6 years.

Restricted Stock Units

Certain employees and consultants have been awarded restricted stock units with time-based vesting. The following table summarizes restricted stock units' activities for the three months ended March 31, 2021:

	Number of Units	Weighted Average Grant Date Fair Value
Nonvested at December 31, 2020	1,468,559	$ 3.87
Granted	589,250	3.46
Forfeited	(45,750)	4.15
Vested	(168,188)	4.96
Nonvested at March 31, 2021	1,843,871	$ 3.63

As of March 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 the remaining weighted average vesting period of approximately1.9 years.

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

	For the three months ended March 31,	
	2021	2020
General and administrative	$ 324	$ 352
Research and development	672	453
Total stock-based compensation expense	$ 996	$ 805

Employee Stock Purchase Plan

Eligible employees can purchase the Company's Common Stock at the end of a predetermined offering period at85% of the lower of the fair market value at the beginning or end of the offering period. The Employee Stock Purchase Plan ("ESPP") is compensatory and results in stock-based compensation expense.

For the three months ended March 31, 2021 and 2020,55,963 and 68,351 shares, respectively, have been purchased and 203,181 shares are available for future sale under the Company's ESPP. The Company received proceeds of $0.2 million and $0.2 million for the three months ended March 31, 2021 and 2020, respectively.

Warrants

A summary of warrant activities for the three months ended March 31, 2021 is presented below:

	Warrants	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (in years)
Outstanding as of December 31, 2020	5,402,670	$ 8.21	1.39
Cashless exercised	(138)	—	—
Outstanding as of March 31, 2021	5,402,532	$ 8.21	1.14

Upon the cashless exercise of warrants, the Company will issue new shares of common stock.

Note 9 – 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 options and warrants, outstanding Class A preferred shares, and unvested restricted stock and restricted stock units 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 three months ended March 31,	
	2021	2020
Warrants	5,402,532	5,402,670
Options	1,141,675	1,241,675
Class A Preferred Shares	250,000	250,000
Unvested restricted stock awards	302,114	299,060
Unvested restricted stock units	1,843,871	980,667
Total	8,940,192	8,174,072

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

Forward-Looking Statements

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with our financial statements and the related notes included elsewhere in this Form 10-Q. Our financial statements have been prepared in accordance with U.S. GAAP. The following discussion and analysis contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934 (the "Exchange Act"), including, without limitation, statements regarding our expectations, beliefs, intentions or future strategies that are signified by the words "expect," "anticipate," "intend," "believe," "may," "plan", "seek" or similar language. All forward-looking statements included in this document are based on information available to us on the date hereof, and we assume no obligation to update any such forward-looking statements. Our business and financial performance are subject to substantial risks and uncertainties. Actual results could differ materially from those projected in the forward-looking statements. In evaluating our business, you should carefully consider the information set forth under the heading "Risk Factors" herein.

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 therapy programs 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 therapy program, 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 for our CAR T therapies we have partnered with the City of Hope National Medical Center ("COH"), Fred Hutchinson Cancer Research Center ("Fred Hutch") and Nationwide Children's Hospital ("Nationwide").

Gene Therapy

In partnership with St. Jude and the National Institutes of Health ("NIH"), our gene therapy program is being conducted under an exclusive license to develop a potentially curative treatment for XSCID, a rare genetic immune system condition also known as bubble boy disease 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 NIH.

In May 2020, we submitted an Investigational New Drug ("IND") application with the U.S. Food and Drug Administration ("FDA") to initiate a registrational multicenter Phase 2 clinical trial of MB-107 in newly diagnosed infants with XSCID who are under the age of two. In response, the FDA identified Chemistry, Manufacturing, and Control ("CMC") hold issues that the Company satisfactorily addressed in a December 2020 submission to the FDA, and the CMC hold was removed in January 2021. The trial is expected to enroll 10 patients who, together with 15 patients enrolled in the current multicenter trial led by St. Jude, will be compared with 25 matched historical control patients who have undergone hematopoietic stem cell transplant ("HSCT"). The primary efficacy endpoint will be event-free survival, and we are targeting topline data from the trial in the second half of 2022.

We further expect to file an IND in the second quarter of 2021 for a registrational multicenter Phase 2 clinical trial of lentiviral gene therapy in previously transplanted XSCID patients (MB-207). We anticipate enrolling 20 patients, and we are targeting topline data for this trial in the first half 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 therapies, support preclinical and clinical research activities by our partners and transfer the underlying manufacturing technology to our cell processing facility located in Worcester, Massachusetts, 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 multicenter Phase 1/2 clinical trial of MB-102, and our clinical trial has begun enrollment for the treatment of patients with blastic plasmacytoid dendritic cell neoplasm ("BPDCN"). On May 10, 2021, the FDA approved our IND application to initiate a multicenter Phase 1/2 clinical trial of MB-106 for the treatment of patients with non-Hodgkin lymphoma and chronic lymphocytic leukemia, and in the third quarter of 2021 we expect to enroll the first patient in this trial. We plan to file an IND application 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 prostate stem cell antigen ("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 fourth quarter of 2021, we plan to file an IND application for the combination of MB-101 and MB-108 for the treatment of patients with GBM. 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. 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). We also plan to file IND applications 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.

Recent Events

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

MB-106 targets CD20, a commercially validated target on the surface of B-cell malignancies such as non-Hodgkin lymphoma ("NHL") and chronic lymphocytic leukemia ("CLL") – cancers that lack a strong CAR T-based clinical focus in the U.S. MB-106 cells express a third-generation CAR derived from a fully human antibody that originated in the Fred Hutch laboratories of the late Oliver Press, M.D., Ph.D., and Brian Till, M.D., Associate Professor in the Clinical Research Division. The CAR T therapy was exclusively licensed to Mustang in 2017, and Fred Hutch and Mustang collaborated to develop the cell processing that will be used in the Mustang IND Phase 1/2 clinical trial.

To date, the same vector planned for use in the manufacturing of MB-106 is currently being evaluated in the ongoing Phase 1/2 study sponsored by Fred Hutch, where Mazyar Shadman, M.D., M.P.H., Associate Professor in the Clinical Research Division, is the principal investigator. Data from this ongoing study were presented by Dr. Shadman at the 62nd Annual American Society of Hematology meeting in 2020 and demonstrated a favorable safety profile in patients with NHL, with an 89% overall response rate (ORR; 8/9 patients) and a 44% complete response rate (CR; 4/9 patients).

On May 10, 2021, we announced that that the FDA has accepted the Company's IND application to initiate a Phase 1/2 multicenter, study to assess the safety, tolerability and efficacy of MB-106.

On May 12, 2021, we announced that interim data from the ongoing Fred Hutch-sponsored Phase 1/2 clinical trial investigating the safety and efficacy of MB-106 have been selected for an e-poster presentation at the European Hematology Association 2021 Virtual Congress ("EHA2021").

In the abstract posted on the EHA2021 website, Fred Hutch reported on 12 patients treated with MB-106, which underwent a major cell manufacturing modification after treating 7 patients with no objective responses as previously reported at the 62nd Annual American Society of Hematology meeting in 2020. The 12 patients were treated at dose levels ("DL") ranging from $3.3x10^5$ to $1x10^7$ CAR T cells/kg, and clinical responses were observed at all DLs with no dose-limiting toxicities. Cytokine release syndrome occurred in 3 patients (25%): 2 patients with grade 1 and 1 patient with grade 2. Only 1 patient required tocilizumab and dexamethasone, and no immune effector cell-associated neurotoxicity syndrome of any grade was observed. Overall response rate ("ORR") was 92% (11/12) with a complete response ("CR") rate of 58% (7/12). In 9 patients with follicular lymphoma, ORR and CR were 89% (8/9) and 67% (6/9), respectively. The patient with CLL had a PET-negative CR and undetectable measurable residual disease in peripheral blood and bone marrow by flow cytometry (10^{-4}) (uMRD4) on day 28. Among patients who received the highest two dose levels, DL3 ($3.3x10^6$ CAR T cells/kg; n=4) and DL4 ($1x10^7$ CAR T cells/kg; n=1), CR rate was 100% (5/5). All 7 patients who achieved a CR remain in remission at a median follow-up of 4 months. CAR T expansion was robust, with median peak blood levels of CAR+ T cells of 122 CAR+ cells/µl (range 0.27-2024), corresponding to 19% (range 0.15 - 65%) of all CD3+ cells. Updated data will be presented at EHA2021.

MB-107 (Ex vivo Lentiviral Therapy for X-linked Severe Combined Immunodeficiency (XSCID) in newborn patients)

In February 2021, we announced that the FDA removed the clinical hold on the MB-107 pivotal Phase 2 clinical trial IND application after reviewing a comprehensive CMC package that was submitted in late December 2020. The Company is proceeding with its plans to initiate the pivotal Phase 2 trial in newly diagnosed XSCID patients.

The same lentiviral vector used in MB-107 is currently being assessed in a Phase 1/2 clinical trial for XSCID in newly diagnosed infants under the age of two at St. Jude, UCSF Benioff Children's Hospital in San Francisco and Seattle Children's Hospital. Additionally, it is being assessed in a Phase 1/2 clinical trial at the National Institute of Allergy and Infectious Diseases ("NIAID"), part of the NIH, for XSCID patients who have been previously treated with HSCT and for whom re-treatment is indicated.

Data from the Phase 1/2 clinical trial led by St. Jude that were presented at the 61st American Society of Hematology ("ASH") Annual Meeting in December 2019 included 11 newly diagnosed XSCID patients who had been treated with a median follow-up at data cut-off of 23.6 months (range 1.5 to 33.9 months). No serious adverse events related to treatment were reported other than hematologic ones related to low-dose busulfan conditioning. Nine patients, with 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 treatment. Five patients were off intravenous immunoglobulin ("IVIG") therapy, of whom 3 responded to vaccines.

To date, all 11 patients have continued to do well and, as also announced in February 2021, 5 additional patients were 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. Enrollment will continue at all three clinical sites until Mustang initiates its multicenter pivotal Phase 2 trial of MB-107.

In September 2020, *The Journal of Allergy and Clinical Immunology: In Practice* published a case study of one patient with XSCID and disseminated Bacille Calmette-Guérin ("BCG") infection, who was enrolled in the clinical trial at St. Jude. After 2.5 years of treatment, the patient has remained clinically well with a stable, functional immune system and protective vaccine titers, despite the complication of the disseminated BCG infection.

MB-207 (Ex vivo Lentiviral Therapy for X-linked Severe Combined Immunodeficiency (XSCID) in previously transplanted patients)

Also in February 2021, we updated results from the ongoing Phase 1/2 clinical trial being conducted by the NIH in older XSCID patients, all of whom had previously received haplo-identical HSCT as infants and were subsequently noted to be experiencing declining immune function with symptomatic infections. At the time of the most recent NIH data presentation at ASH in 2019, 8 patients had been treated without transduction enhancers (referred to as Cohort A) and had been followed for 3 to 7 years. Seven of these 8 patients experienced gradual clinical benefit in terms of clearance of chronic norovirus and associated improved abdominal complaints, malabsorption, growth and IgG production. One of these 7 patients died 27 months after gene therapy of a pulmonary bleed related to chronic bronchiectasis that predated the therapy and was deemed to be unrelated to therapy.

In an attempt to address the relatively slow resolution of chronic norovirus observed in most of these 7 patients and the delayed immune cell recovery and persistent clinical disease in the eighth patient, transduction enhancers were introduced in the cell processing for the subsequent 6 patients (referred to as Cohort B), which included retreatment of the eighth patient in Cohort A who had delayed immune recovery and persistent clinical disease. This enhanced transduction 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 a result, Mustang has licensed Sirion Biotech's Lentiboost™ and will include transduction enhancement in its pivotal Phase 2 clinical trial for MB-207 in this patient population.

To date, 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 have been able to discontinue IVIG and have experienced sustained restoration of humoral responses to immunization. The remaining 3 patients have had reduced IVIG requirements. All chronic norovirus infections have resolved, and the quality of life of all patients has improved significantly.

The original 6 patients in Cohort B also continue to do well, with longest follow-up now 22 months. Two additional patients have been 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. Further enrollment at NIH is now limited pending Mustang's initiation of its pivotal multicenter Phase 2 clinical trial, and the company expects to submit an IND application for this trial in the second quarter of 2021.

At-the-Market Offering

During the three months ended March 31, 2021, we issued approximately 11.6 million shares of common stock at an average price of $4.17 per share for gross proceeds of $48.4 million under the At-the-Market Issuance Sales Agreement (the "Mustang ATM") with B. Riley Securities, Inc., Cantor Fitzgerald & Co., National Securities Corporation, Oppenheimer & Co. Inc. and H.C. Wainwright & Co., LLC (each an "Agent" and collectively, the "Agents"). In connection with these sales, we paid aggregate fees of approximately $0.9 million for net proceeds of approximately $47.5 million.

Registration Statement

On April 23, 2021, the Company filed a shelf registration statement on Form S-3 (the "2021 S-3"), which has not yet been declared effective. Under the 2021 S-3, the Company may sell up to a total of $200.0 million of its securities upon being declared effective.

Critical Accounting Policies and Use of Estimates

See Note 2 to our Financial Statements.

Results of Operations

Comparison of the Three Months Ended March 31, 2021 and 2020

	For the three months ended March 31,		Change	
($in thousands)	2021	2020	$	%
Operating expenses:				
Research and development	$ 11,618	$ 9,314	$ 2,304	25 %
Research and development – licenses acquired	—	250	(250)	(100)%
General and administrative	3,469	1,956	1,513	77 %
Total operating expenses	15,087	11,520	3,567	31 %
Loss from operations	(15,087)	(11,520)	(3,567)	31 %
Other income (expense)				
Interest income	134	263	(129)	(49)%
Interest expense	(4)	(600)	596	(99)%
Total other income (expense)	130	(337)	467	(139)%
Net Loss	**$ (14,957)**	**$ (11,857)**	**$ (3,100)**	**26 %**

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.

For the three months ended March 31, 2021 and 2020, research and development expenses were $11.6 million and $9.3 million, respectively. The increase of approximately $2.3 million is primarily due to $1.2 million for laboratory supplies, $0.8 million for consulting and professional fees, $0.5 million for sponsored research and clinical trial agreements, $0.4 million for personnel related expenses, $0.2 million for stock-based compensation and, partially offset by third party clinical trial costs of $1.0 million.

For the three months ended March 31, 2021 and 2020, research and development expenses for licenses acquired were approximately $0 million and $0.3 million, respectively. For the three months ended March 31, 2020, we expensed $0.3 million related to our HER2 license with COH.

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;
- 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.

For the three months ended March 31, 2021 and 2020, general and administrative expenses were $3.5 million and $2.0 million, respectively. The increase of approximately $1.5 million is primarily attributed to $0.9 million of expense for the shares issued to Fortress, which equaled 2.5% of the gross proceeds to the At-the-Market offering completed during the three months ended March 31, 2021.

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 under Mustang's INDs;
- 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 or Expense

For the three months ended March 31, 2021 and 2020, other income (expense) was $0.1 million and ($0.3) million, respectively. The decrease of approximately $0.4 million in expense is primarily attributed to $0.6 million lower interest expense related to the prepayment of the Horizon Notes and lower interest income of approximately $0.1 million.

Liquidity and Capital Resources

We have incurred substantial operating losses and expect to continue to incur significant operating losses for the foreseeable future and may never become profitable. As of March 31, 2021, we had an accumulated deficit of $200.4 million.

We have funded our operations to date primarily through the sale of equity. We expect to continue to use the proceeds from previous financing transactions primarily for general corporate purposes, including financing our growth, developing new or existing product candidates, and funding capital expenditures, acquisitions and investments. We currently anticipate that our cash and cash equivalents balances at March 31, 2021, are sufficient to fund our anticipated operating cash requirements for at least one year from the filing date of this Form 10-Q.

We will be required to expend significant funds in order to advance the development of our product candidates. We 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 our existing and any new product candidates. In addition to the foregoing, based on our current assessment, we do not expect any material impact on our long-term development timeline and our liquidity due to the worldwide spread of the COVID-19 virus. However, we are continuing to assess the effect on our operations by monitoring the spread of COVID-19 and the actions implemented to combat the virus throughout the world.

Cash Flows for the Three months Ended March 31, 2021 and 2020

($in thousands)	For the three months ended March 31, 2021	2020
Statement of cash flows data:		
Total cash (used in) provided by:		
Operating activities	$ (15,580)	$ (10,152)
Investing activities	(458)	(526)
Financing activities	47,605	5,079
Net change in cash, cash equivalents and restricted cash	$ 31,567	$ (5,599)

Operating Activities

Net cash used in operating activities was $15.6 million for the three months ended March 31, 2021, compared to $10.2 million for the three months ended March 31, 2020.

Net cash used in operating activities for the three months ended March 31, 2021, was primarily due to approximately $15.0 million in net loss and $3.3 million in change in operating assets and liabilities, partially offset by $1.2 million of equity fee on issuance of common shares to Fortress Biotech, $1.0 million of non-cash stock compensation expenses and $0.5 million of depreciation.

Net cash used in operating activities for the three months ended March 31, 2020, was primarily due to approximately $11.9 million in net loss, partially offset by $0.8 million of non-cash stock compensation expenses, $0.4 million of depreciation, $0.3 million of research and development-licenses acquired, $0.3 million of accretion of debt discount and $0.1 million of equity fee on issuance of common shares to Fortress Biotech.

Investing Activities

Net cash used in investing activities was $0.5 million and $0.5 million for the three months ended March 31, 2021 and 2020, respectively, representing purchases of fixed assets.

Financing Activities

Net cash provided by financing activities was $47.6 million during the three months ended March 31, 2021, due to gross proceeds of $48.4 million, net of offering costs of $0.9 million, from the Mustang ATM; and $0.2 million raised from the issuance of the Company's common shares in connection with the ESPP.

Net cash provided by financing activities was $5.1 million during the three months ended March 31, 2020, due to gross proceeds of $5.0 million, net of offering costs of $0.1 million, from the Mustang ATM and $0.2 million raised from the issuance of the Company's common shares in connection with the ESPP.

Off-Balance Sheet Arrangements

We are not party to any off-balance sheet transactions. We have no guarantees or obligations other than those which arise out of normal business operations.

Item 3. Quantitative and Qualitative Disclosures About Market Risks

During the three months ended March 31, 2021, there were no material changes to our interest rate risk disclosures, market risk disclosures or foreign currency exchange rate risk disclosures reported in our Annual Report on Form 10-K for the year ended December 31, 2020.

Item 4. Controls and Procedures

Disclosure Controls and Procedures

We maintain "disclosure controls and procedures," as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), that are designed to ensure that information required to be disclosed by us in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in Securities and Exchange Commission rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and our Principal Financial Officer, to allow timely decisions regarding required disclosure.

The design of any disclosure controls and procedures also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions.

With respect to the quarter ended March 31, 2021, under the supervision and with the participation of our management, we conducted an evaluation of the effectiveness of the design and operations of our disclosure controls and procedures. Based upon this evaluation, the Company's Chief Executive Officer and Principal Financial Officer have concluded that the Company's disclosure controls and procedures are effective.

Management does not expect that our internal control over financial reporting will prevent or detect all errors and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control systems are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in a cost-effective control system, no evaluation of internal control over financial reporting can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud, if any, have been or will be detected.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the fiscal quarter ended March 31, 2021, which have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

None

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-Q 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-Q, 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. Additionally, many of these risks and uncertainties are currently elevated by and may or will continue to be elevated by the COVID-19 pandemic.

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 $200.4 million as of March 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 our 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 our 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 March 31, 2021, we had $130.4 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 Agreement with Horizon, which we entered into in March 2019, 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, to Horizon. 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 Horizon's lien on our assets, as determined by Horizon, 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 Horizon, 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 our 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 regulate 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 our 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, the developing situation in China and globally regarding the coronavirus disease outbreak has 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, and the University of Alabama at Birmingham 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, and UAB 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 laboratory team at COH, of Dr. Brian Till and his laboratory team at Fred Hutch, of Drs. Stephen Gottschalk and Ewelina Mamcarz at St. Jude, and of Dr. James M. Markert at UAB. 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 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 has caused consideration 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. Back in March, Dr. Janet Woodcock, the Director of the FDA's Center for Drug Evaluation and Research, temporarily stepped away from her role to focus on the therapeutic aspects of Operation Warp Speed, a major reorganization intended to better align the FDA's activities with the national effort to develop COVID-19 countermeasures. Dr. Woodcock was later named Acting Commissioner of the FDA on January 20, 2021. These changes to leadership, enhanced focus on COVID-19 countermeasures, and the reorganization and rededication or critical resources, both at the FDA and within similar governmental authorities across the world, are likely to 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 are implementing 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 has 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 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 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 the coronavirus.

The Company's employees and consultants are being affected by the COVID-19 pandemic. Substantially all of our office and management personnel are working remotely, and the Company may need to enact further precautionary measures to help minimize the risk of our employees being exposed to the coronavirus. COVID-19 may also compromise the ability of independent contractors who perform consulting services for us to deliver services or deliverables in a satisfactory or timely manner. Further, our management team is focused on mitigating the adverse effects of the COVID-19 pandemic, which has required and will continue to require 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.

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 emerging 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 2. Unregistered Sales of Equity Securities and Use of Proceeds

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

None.

Item 5. Other Information

None.

Item 6. Exhibits

The exhibits listed on the Exhibit Index are either filed or furnished with this report or incorporated herein by reference.

EXHIBIT INDEX

Exhibit No.	Description
31.1	Certification of President and Chief Executive Officer (Principal Executive Officer), pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 (filed herewith).
31.2	Certification of Senior Vice President of Finance & Corporate Controller (Principal Financial Officer), pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 (filed herewith).
32.1	Certification of President and Chief Executive Officer (Principal Executive Officer), pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 (filed herewith).
32.2	Certification of Senior Vice President of Finance & Corporate Controller (Principal Financial Officer), pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 (filed herewith).
101	The following financial information from the Company's Quarterly Report on Form 10-Q for the period ended March 31, 2021, formatted as Inline Extensible Business Reporting Language (iXBRL): (i) the Condensed Balance Sheets, (ii) the Condensed Statements of Operations, (iii) the Condensed Statement of Stockholders' Equity, (iv) the Condensed Statements of Cash Flows, and (v) Notes to the Condensed Financial Statements (filed herewith).
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in exhibit 101)

Signatures

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

MUSTANG BIO, INC.

May 14, 2021

By: /s/ Manuel Litchman
Manuel Litchman, M.D., President and
Chief Executive Officer
(Principal Executive Officer)

By: /s/ Brian Achenbach
Brian Achenbach
Senior Vice President of Finance & Corporate Controller
(Principal Financial Officer)

Exhibit 31.1

MUSTANG BIO, INC.
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 Quarterly Report on Form 10-Q 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, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b. 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 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 this report any change in the Registrant's internal control over financial reporting that occurred during the Registrant's most recent fiscal quarter (the Registrant's fourth 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 controls over financial reporting.

May 14, 2021

By: /s/ Manuel Litchman
Manuel Litchman, M.D., President and
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

MUSTANG BIO, INC.
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 Quarterly Report on Form 10-Q 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, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b. 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 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 this report any change in the Registrant's internal control over financial reporting that occurred during the Registrant's most recent fiscal quarter (the Registrant's fourth 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 controls over financial reporting.

May 14, 2021

By: /s/ Brian Achenbach
Brian Achenbach
Senior Vice President of Finance & Corporate Controller
(Principal Financial Officer)

Exhibit 32.1

MUSTANG BIO, INC.
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 Quarterly Report of Mustang Bio, Inc. (the "Company") on Form 10-Q for the quarterly period ended March 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 (Principal 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.

May 14, 2021

By: /s/ Manuel Litchman
Manuel Litchman, M.D., President and
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

MUSTANG BIO, INC.
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 Quarterly Report of Mustang Bio, Inc. (the "Company") on Form 10-Q for the quarterly period ended March 31, 2021, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Brian Achenbach, Senior Vice President of Finance & Corporate Controller (Principal Financial 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.

May 14, 2021

By: /s/ Brian Achenbach
Brian Achenbach
Senior Vice President of Finance & Corporate Controller
(Principal Financial Officer)