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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 September 30, 2022
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: 001-38541

Magenta Therapeutics, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**81-0724163**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
100 Technology Square Cambridge, Massachusetts	**02139**
(Address of principal executive offices)	**(Zip Code)**

(857) 242-0170
(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, $0.001 Par Value	**MGTA**	**The 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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

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

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

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

As of October 31, 2022, there were 60,555,520 shares of Common Stock, $0.001 par value per share, outstanding.

Magenta Therapeutics, Inc.

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RISK FACTOR SUMMARY

The risk factors detailed in Item 1A entitled "Risk Factors" in this Quarterly Report on Form 10-Q are the risks that we believe are material to our investors and a reader should carefully consider them. Those risks are not all of the risks we face and other factors not presently known to us or that we currently believe are immaterial may also affect our business if they occur. The following is a summary of the risk factors detailed in Item 1A:

- We are a clinical stage company with a limited operating history, have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future. We have no products approved for commercial sale and have not generated any revenue from product sales.
- We will require additional capital to fund our operations, and if we fail to obtain necessary financing, we will not be able to complete the development and commercialization of our product candidates. Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.
- Although we have initiated clinical trials for certain of our product candidates, we have not yet demonstrated an ability to successfully complete certain clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial-scale medicine, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization of our product candidates. We have never generated revenue from product sales and may never be profitable.
- We are early in our development efforts for our product candidates. If we are unable to advance our product candidates through development, obtain regulatory approval and commercialize them, or if we experience significant delays in doing so, our business will be materially harmed.
- The successful development of biopharmaceuticals and cell-based therapies is highly uncertain. Our ongoing and planned clinical trials or those of our collaborators involving our product candidates may reveal significant adverse events not seen in our preclinical and clinical studies and may result in a safety profile that could inhibit regulatory approval or market acceptance of any of our product candidates.
- Stem cell transplant is a high-risk procedure with curative potential that may result in complications or adverse events for patients in our clinical trials or for patients that use any of our product candidates, if approved.
- If we are not able to identify a safe and effective dose for any of our product candidates, including our antibody drug conjugates, or ADCs, such as MGTA-117 utilizing an amanitin toxin not previously tested in humans, we may need to delay, abandon or limit our development of any potential product candidates.
- Clinical development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of any product candidates. If we encounter delays or difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.
- The results of earlier studies and interim data from our ongoing studies may not be predictive of future clinical trial results, and we may fail to establish an adequate safety or efficacy profile to conduct advanced clinical trials or obtain regulatory approval for our product candidates.
- If we are unable to successfully develop our current programs into a comprehensive portfolio of product candidates, or experience significant delays in doing so, we may not realize the full commercial potential of our current and future product candidates. We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.
- We have no experience as a company in obtaining regulatory approval for a drug or biologic. Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate we may develop, and any such approval may be for a narrower indication than we seek.
- Because we are developing product candidates for the treatment of diseases in which there is little clinical experience using new technologies, there is increased risk that the U.S. Food and Drug Administration, or FDA, the European Medicines Agency, or EMA, or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results, and these results may be difficult to analyze.
- Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate we may develop, and any such approval may be for a narrower indication than we seek.

- We rely on third parties to conduct our preclinical and clinical trials, process and product development and current Good Manufacturing Practices, or cGMP, and we will rely on them to perform other tasks for us. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.
- We currently rely, and expect to continue to rely, on third parties to manufacture our clinical product supplies, and we intend to rely on third parties to produce and process our product candidates, if approved. This reliance increases the risk that we may not have sufficient quantities of our product candidates or may not be able to produce such quantities at an acceptable cost or quality level, which could delay, prevent or impair our development or commercialization efforts.
- Any contamination in our or our third parties' manufacturing process, shortages of raw materials or reagents or failure of any of our key suppliers to deliver necessary components of our product candidates could result in delays in our clinical development or marketing schedules.
- We may never obtain FDA approval for any of our product candidates in the U.S., and even if we do, we may never obtain approval for or commercialize any of our product candidates in any other jurisdiction, which would limit our ability to realize their full market potential.
- Even if our product candidates are approved by government regulators, the commercial success of any of our product candidates will depend upon the degree of market acceptance by physicians, patients, third-party payors and others in the medical community. Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, if approved, which could make it difficult for us to sell any product candidates or therapies profitably.
- We currently have no marketing and sales organization and have no experience in marketing products. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be able to generate product revenue.
- We face substantial competition, including from companies with greater financial, technical, research, manufacturing, marketing, distribution and other resources than us, which may result in others discovering, developing or commercializing products before or more successfully than we do.
- We are highly dependent on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.
- It is difficult and costly to protect our intellectual property and our proprietary technologies, and we may not be able to ensure their protection. If we are unable to obtain and maintain sufficient intellectual property protection for our product candidates, or our technologies, we may not be able to compete effectively in our markets and our business may be adversely affected.
- We currently depend, and may in the future continue to depend, on collaborations with third parties for the research, development, and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates and our business may be adversely affected.
- We may not be successful in finding strategic collaborators for continuing development of certain of our product candidates or successfully commercializing or competing in the market for certain indications. If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.
- The coronavirus, or COVID-19, pandemic or any future pandemic, epidemic or outbreak of any other highly infectious disease could have a material adverse effect on our business, financial condition and results of operations.
- If we lose key personnel, or if we fail to recruit additional highly skilled personnel, our ability to develop our product candidates will be impaired and our business may be harmed.
- The trading price of our common stock has been, and will likely continue to be, highly volatile. As a result of this volatility, investors may not be able to sell common stock at or above the purchase price and may lose some or all of their investment.

This section contains forward-looking statements. You should refer to the explanation of the qualifications and limitations on forward-looking statements in this Quarterly Report on Form 10-Q.

PART I—FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS.

Magenta Therapeutics, Inc.

Consolidated Balance Sheets
(In thousands, except share and per share data)
(Unaudited)

	September 30, 2022	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 59,028	$ 131,650
Marketable securities	69,256	45,276
Prepaid expenses and other current assets	3,813	3,767
Total current assets	132,097	180,693
Restricted cash	1,780	1,780
Operating lease, right-of-use asset	23,930	—
Property and equipment, net	6,316	7,461
Total assets	$ 164,123	$ 189,934
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,578	$ 3,040
Accrued expenses and other current liabilities	6,722	7,823
Operating lease liability, current portion	3,621	—
Total current liabilities	12,921	10,863
Operating lease liability, net of current portion	27,177	—
Deferred rent	—	6,399
Total liabilities	40,098	17,262
Commitments and contingencies (Note 7)		
Stockholders' Equity:		
Preferred stock, $0.001 par value; 10,000,000 shares authorized; no shares issued or outstanding	—	—
Common stock, $0.001 par value; 150,000,000 shares authorized; 60,555,520 and 58,799,157 shares issued and outstanding as of September 30, 2022 and December 31, 2021, respectively	61	59
Additional paid-in capital	506,277	498,210
Accumulated other comprehensive loss	(462)	(30)
Accumulated deficit	(381,851)	(325,567)
Total stockholders' equity	124,025	172,672
Total liabilities and stockholders' equity	$ 164,123	$ 189,934

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

Magenta Therapeutics, Inc.

Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 11,201	$ 10,795	$ 39,351	$ 33,652
General and administrative	6,052	7,450	19,819	20,900
Total operating expenses	17,253	18,245	59,170	54,552
Loss from operations	(17,253)	(18,245)	(59,170)	(54,552)
Interest and other income, net	1,190	818	2,886	2,708
Net loss	$ (16,063)	$ (17,427)	$ (56,284)	$ (51,844)
Net loss per share, basic and diluted	$ (0.27)	$ (0.30)	$ (0.95)	$ (0.97)
Weighted average common shares outstanding, basic and diluted	59,269,965	58,583,476	58,963,280	53,655,314
Comprehensive loss:				
Net loss	$ (16,063)	$ (17,427)	$ (56,284)	$ (51,844)
Other comprehensive income (loss):				
Unrealized gains (losses) on marketable securities	140	(1)	(432)	24
Total other comprehensive income (loss)	140	(1)	(432)	24
Total comprehensive loss	$ (15,923)	$ (17,428)	$ (56,716)	$ (51,820)

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

Magenta Therapeutics, Inc.

Consolidated Statements of Stockholders' Equity
(In thousands, except share data)
(Unaudited)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
	Three Months Ended September 30, 2022					
Balances at June 30, 2022	58,848,861	$ 59	$ 502,009	$ (602)	$ (365,788)	$ 135,678
Issuance of common stock under the ATM Program, net of commissions and offering costs	1,644,200	2	2,761	—	—	2,763
Vesting of restricted stock	62,459	—	—	—	—	—
Stock-based compensation expense	—	—	1,507	—	—	1,507
Unrealized gains on marketable securities	—	—	—	140	—	140
Net loss	—	—	—	—	(16,063)	(16,063)
Balances at September 30, 2022	60,555,520	$ 61	$ 506,277	$ (462)	$ (381,851)	$ 124,025
	Three Months Ended September 30, 2021					
Balances at June 30, 2021	58,508,735	$ 59	$ 491,679	$ 2	$ (288,848)	$ 202,892
Vesting of restricted stock	209,998	—	—	—	—	—
Issuance of common stock upon exercise of stock options	58,538	—	425	—	—	425
Stock-based compensation expense	—	—	3,799	—	—	3,799
Unrealized losses on marketable securities	—	—	—	(1)	—	(1)
Net loss	—	—	—	—	(17,427)	(17,427)
Balances at September 30, 2021	58,777,271	$ 59	$ 495,903	$ 1	$ (306,275)	$ 189,688

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

Magenta Therapeutics, Inc.

Consolidated Statements of Stockholders' Equity (Continued)
(In thousands, except share data)
(Unaudited)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
	Nine Months Ended September 30, 2022					
Balances at December 31, 2021	58,799,157	$ 59	$ 498,210	$ (30)	$ (325,567)	$ 172,672
Issuance of common stock under the ATM Program, net of commissions and offering costs	1,644,200	2	2,761	—	—	2,763
Issuance of common stock under Employee Stock Purchase Plan	49,704	—	49	—	—	49
Vesting of restricted stock	62,459	—	—	—	—	—
Stock-based compensation expense	—	—	5,257	—	—	5,257
Unrealized losses on marketable securities	—	—	—	(432)	—	(432)
Net loss	—	—	—	—	(56,284)	(56,284)
Balances at September 30, 2022	60,555,520	$ 61	$ 506,277	$ (462)	$ (381,851)	$ 124,025
	Nine Months Ended September 30, 2021					
Balances at December 31, 2020	48,533,135	$ 49	$ 398,311	$ (23)	$ (254,431)	$ 143,906
Issuance of common stock upon private investment, net of offering costs	9,599,998	10	86,087	—	—	86,097
Vesting of restricted stock	218,464	—	—	—	—	—
Issuance of common stock upon exercise of stock options	411,685	—	3,294	—	—	3,294
Issuance of common stock under Employee Stock Purchase Plan	13,989	—	86	—	—	86
Stock-based compensation expense	—	—	8,125	—	—	8,125
Unrealized gains on marketable securities	—	—	—	24	—	24
Net loss	—	—	—	—	(51,844)	(51,844)
Balances at September 30, 2021	58,777,271	$ 59	$ 495,903	$ 1	$ (306,275)	$ 189,688

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

Magenta Therapeutics, Inc.

Consolidated Statements of Cash Flows
(In thousands)
(Unaudited)

	Nine months ended September 30,	
	2022	2021
Cash flows from operating activities:		
Net loss	$ (56,284)	$ (51,844)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	5,257	8,125
Depreciation and amortization expense	1,459	1,485
Loss on disposal of property and equipment	—	95
Noncash lease expense	2,158	—
Net amortization of premiums on marketable securities	306	630
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(46)	(1,167)
Accounts payable	(462)	(1,874)
Accrued expenses and other current liabilities	(546)	591
Operating lease liabilities	(2,244)	—
Deferred rent	—	118
Net cash used in operating activities	(50,402)	(43,841)
Cash flows from investing activities:		
Purchases of property and equipment	(314)	(1,249)
Purchases of marketable securities	(59,718)	—
Maturities of marketable securities	35,000	55,000
Net cash provided by (used in) investing activities	(25,032)	53,751
Cash flows from financing activities:		
Proceeds from private investment	—	86,400
Proceeds from issuance of common stock under the ATM Program, net of commissions	2,904	—
Payments of offering costs	(141)	(303)
Proceeds from exercise of common stock options	—	3,294
Proceeds from issuance of common stock under Employee Stock Purchase Plan	49	86
Net cash provided by financing activities	2,812	89,477
Net increase (decrease) in cash, cash equivalents and restricted cash	(72,622)	99,387
Cash, cash equivalents and restricted cash at beginning of period	133,430	59,932
Cash, cash equivalents and restricted cash at end of period	$ 60,808	$ 159,319

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

Magenta Therapeutics, Inc.

Notes to Consolidated Financial Statements
(Unaudited)

1. Nature of the Business and Basis of Presentation

Magenta Therapeutics, Inc. (the "Company") is a clinical-stage biotechnology company developing novel medicines designed to bring the curative power of stem cell transplant to more patients with blood cancers, genetic diseases and autoimmune diseases. The Company was incorporated under the laws of the State of Delaware in June 2015 as HSCTCo Therapeutics, Inc. In February 2016, the Company changed its name to Magenta Therapeutics, Inc. and in June 2018 the Company completed an initial public offering of its common stock.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations, the continuing impact of the ongoing coronavirus ("COVID-19") pandemic and the ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

The Company has a shelf registration statement on Form S-3 (the "Shelf") on file with the SEC, which covers the offering, issuance and sale of up to an aggregate of $250.0 million of common stock, preferred stock, debt securities, warrants and/or units of any combination thereof. The Company also entered into a sales agreement, as amended, with Cowen and Company, LLC, as sales agent to provide for the issuance and sale by the Company of up to $50.0 million of common stock from time to time in "at-the-market" offerings under the Shelf (the "ATM Program"). The Shelf was declared effective by the SEC on August 12, 2022. During the nine months ended September 30, 2022, the Company sold 1,644,200 shares of its common stock under the ATM program at a weighted average price per share of $1.82 resulting in net proceeds of $2.8 million after commissions and offering costs. As of September 30, 2022, $247.0 million remained available under the Shelf, including up to $47.0 million available for sale under the ATM Program.

The Company has incurred recurring losses since inception, including net losses of $56.3 million for the nine months ended September 30, 2022 and $71.1 million the year ended December 31, 2021. As of September 30, 2022, the Company had an accumulated deficit of $381.9 million. The Company expects to continue to generate operating losses for the foreseeable future. The Company expects that its cash, cash equivalents and marketable securities will be sufficient to fund its operating expenses and capital expenditure requirements through at least 12 months from the issuance date of these consolidated financial statements. The future viability of the Company beyond that point is dependent on its ability to raise additional capital to fund its operations.

The Company will need to obtain substantial additional funding in connection with continuing operations, particularly as the Company advances its preclinical activities and clinical trials for its product candidates in development. If the Company is unable to raise capital when needed, or on attractive terms, it could be forced to delay, reduce or eliminate its research or drug development programs or any future commercialization efforts. Although management continues to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

The consolidated financial statements include the accounts of the Company and its wholly owned subsidiary. All intercompany balances and transactions have been eliminated. The consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these

financial statements include, but are not limited to, the accrual for research and development expenses and the valuation of stock-based awards. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from those estimates.

Unaudited Interim Financial Information

The consolidated balance sheet at December 31, 2021 was derived from audited financial statements but does not include all disclosures required by GAAP. The accompanying unaudited consolidated financial statements as of September 30, 2022 and for the three and nine months ended September 30, 2022 and 2021 have been prepared by the Company pursuant to the rules and regulations of the SEC for interim financial statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. The Company believes, however, that the disclosures are adequate to make the information presented not misleading. These consolidated financial statements should be read in conjunction with the Company's audited financial statements for the year ended December 31, 2021 included in the Company's Annual Report on Form 10-K, on file with SEC. In the opinion of management, all adjustments, consisting only of normal recurring adjustments necessary for a fair statement of the Company's consolidated financial position as of September 30, 2022 and consolidated results of operations for the three and nine months ended September 30, 2022 and 2021 and consolidated cash flows for the nine months ended September 30, 2022 and 2021 have been made. The results of operations for the nine months ended September 30, 2022 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2022 or any other interim period.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's cash equivalents and marketable securities are carried at fair value, determined according to the fair value hierarchy described above (see Note 3). The carrying values of the Company's accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

Income Taxes

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred taxes are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect in the years in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

Magenta Therapeutics, Inc.

Notes to Consolidated Financial Statements
(Unaudited)

The Company accounts for uncertainty in income taxes recognized in its consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Leases

Prior to January 1, 2022, the Company accounted for leases under ASC 840, *Leases* ("ASC 840"). Effective January 1, 2022, the Company accounts for leases under ASC 842, *Leases* ("ASC 842"). Therefore, as of December 31, 2021 and for the three and nine months ended September 30, 2021, the Company's consolidated financial statements continue to be presented in accordance with ASC 840, the accounting standard originally in effect for such periods. As of and for the three and nine months ended September 30, 2022, the Company's consolidated financial statements are presented in accordance with ASC 842.

In accordance with ASC 842, the Company accounts for a contract as a lease when it has the right to control the asset for a period of time while obtaining substantially all of the asset's economic benefits. The Company determines if an arrangement is a lease or contains an embedded lease at inception. For arrangements that meet the definition of a lease, the Company determines the initial classification and measurement of its right-of-use asset and lease liability at the lease commencement date and thereafter if modified. The lease term includes any renewal options that the Company is reasonably assured to exercise. The present value of lease payments is determined by using the interest rate implicit in the lease, if that rate is readily determinable; otherwise, the Company uses its estimated secured incremental borrowing rate for that lease term. The Company's policy is to not record leases with an original term of twelve months or less on its consolidated balance sheets and recognizes those lease payments in the income statement on a straight-line basis over the lease term. The Company's existing leases are for office and laboratory space.

In addition to rent, the leases may require the Company to pay additional costs, such as utilities, maintenance and other operating costs, which are generally referred to as non-lease components. The Company has elected to not separate lease and non-lease components. Only the fixed costs for lease components and their associated non-lease components are accounted for as a single lease component and recognized as part of a right-of-use asset and lease liability. Rent expense for operating leases is recognized on a straight-line basis over the reasonably assured lease term based on the total lease payments and is included in operating expense in the consolidated statements of operations and comprehensive loss.

Comprehensive Loss

Comprehensive loss includes net loss as well as other changes in stockholders' equity that result from transactions and economic events other than those with stockholders. For the three and nine months ended September 30, 2022 and 2021, the Company's only element of other comprehensive income (loss) was unrealized gains (losses) on marketable securities.

Net Loss per Share

Basic net income (loss) per share is computed by dividing the net income (loss) by the weighted average number of shares of common stock outstanding for the period. Diluted net income (loss) per share is computed by dividing net income (loss) by the weighted average number of common shares outstanding for the period, including potential dilutive common shares assuming the dilutive effect of outstanding stock options. For periods in which the Company has reported net losses, diluted net loss per common share is the same as basic net loss per common share, since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive.

Magenta Therapeutics, Inc.

Notes to Consolidated Financial Statements
(Unaudited)

The Company reported a net loss for the three and nine months ended September 30, 2022 and 2021. The following potential dilutive securities, presented based on amounts outstanding at each period end, have been excluded from the calculation of diluted net loss per share because including them would have had an anti-dilutive impact:

	As of September 30,	
	2022	2021
Stock options to purchase common stock	8,601,539	6,091,220
Unvested restricted common stock units	416,967	497,418
Shares of common stock issuable under Employee Stock Purchase Plan	76,086	8,843
	9,094,592	6,597,481

Recently Issued Accounting Pronouncements

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments – Credit Losses (Topic 326)*. The new standard adjusts the accounting for assets held at amortized costs basis, including marketable securities accounted for as available for sale. The standard eliminates the probable initial recognition threshold and requires an entity to reflect its current estimate of all expected credit losses. The allowance for credit losses is a valuation account that is deducted from the amortized cost basis of the financial assets to present the net amount expected to be collected. For public entities, the guidance was effective for annual reporting periods beginning after December 15, 2019 and for interim periods within those fiscal years. For nonpublic entities and emerging growth companies that choose to take advantage of the extended transition period, the guidance is effective for annual reporting periods beginning after December 15, 2020. Early adoption is permitted for all entities. In November 2019, the FASB issued ASU No. 2019-10, which deferred the effective date for nonpublic entities to annual reporting periods beginning after December 15, 2022, including interim periods within those fiscal years. The Company does not believe the guidance will have a material impact on its consolidated financial statements.

Recently Adopted Accounting Pronouncements

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* ("ASU 2016-02"), which require lessees to recognize most leases on their balance sheet as a right-of-use asset and a lease liability. In general, lease arrangements exceeding a twelve-month term must be recognized as assets and liabilities on the balance sheet. Under ASU 2016-02, a right-of-use asset and lease obligation is recorded for all leases, whether operating or financing, while the income statement reflects lease expense for operating leases and amortization and interest expense for financing leases. The FASB also issued ASU 2018-10, *Codification Improvements to Topic 842 Leases*, and ASU 2018-11, *Targeted Improvements to Topic 842 Leases*, which allows the new lease standard to be applied as of the adoption date with a cumulative-effect adjustment to the opening balance of retained earnings rather than retroactive restatement of all periods presented. The Company adopted the new leasing standards on January 1, 2022 using a modified retrospective approach applied at the beginning of the period of adoption.

The Company elected the "package of practical expedients," which permits the Company not to reassess under the new standards for prior conclusions about lease identification, lease classification and initial direct costs. The Company did not apply the hindsight practical expedient when determining the lease term for existing leases and assessing impairment of expired or existing leases. The Company elected to utilize its incremental borrowing rate based on the remaining lease term as of the date of adoption. In connection with the adoption of ASU 2016-02, the Company recognized a right-of-use asset of $26.1 million and lease liabilities of $33.0 million on its consolidated balance sheet. The deferred rent balance of $7.0 million as of January 1, 2022 was recorded as an offset to the Company's right-of-use asset. The adoption of the standard did not have a material impact on the Company's results of operations or cash flows.

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3. Fair Value of Financial Assets

As of September 30, 2022, marketable securities by security type consisted of (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
U.S. treasury notes (due within one year)	$ 69,718	$ —	$ (462)	$ 69,256
Total	$ 69,718	$ —	$ (462)	$ 69,256

As of December 31, 2021, marketable securities by security type consisted of (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
U.S. treasury notes (due within one year)	$ 30,213	$ —	$ (20)	$ 30,193
U.S. treasury notes (due after one year through two years)	15,093	—	(10)	15,083
Total	$ 45,306	$ —	$ (30)	$ 45,276

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values (in thousands):

	Fair Value Measurements at September 30, 2022 Using:			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 58,026	$ —	$ —	$ 58,026
Marketable securities:				
U.S. treasury notes	—	69,256	—	69,256
Total	$ 58,026	$ 69,256	$ —	$ 127,282

	Fair Value Measurements at December 31, 2021 Using:			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 131,542	$ —	$ —	$ 131,542
Marketable securities:				
U.S. treasury notes	—	45,276	—	45,276
Total	$ 131,542	$ 45,276	$ —	$ 176,818

4. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands):

	September 30, 2022	December 31, 2021
Accrued payroll and related expenses	$ 3,284	$ 3,346
Accrued external research and development expenses	2,617	2,813
Deferred rent, current portion	—	555
Accrued other	821	1,109
	$ 6,722	$ 7,823

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5. Stock-Based Awards

2018 Stock Option and Incentive Plan

The Company grants stock-based awards under the Magenta Therapeutics, Inc. 2018 Stock Option and Incentive Plan (the "2018 Plan"). The Company also has outstanding stock options under the Magenta Therapeutics, Inc. 2016 Stock Option and Grant Plan, as amended (the "2016 Plan"), but is no longer granting awards under the 2016 Plan. As of September 30, 2022, 3,000,865 shares of common stock were available for issuance under the 2018 Plan.

Grant of Stock Options

During the nine months ended September 30, 2022, the Company granted options to certain employees, directors and consultants with service-based vesting conditions for the purchase of 4,357,798 shares of common stock with a weighted average grant date fair value of $1.51 per share. Stock-based compensation expense is being recognized over the requisite service period of 18 months to four years.

Grant of Restricted Stock Units

During the nine months ended September 30, 2022, the Company granted 160,558 restricted stock units to certain employees with a weighted average grant date fair value of $2.01 per share. Stock-based compensation expense is being recognized over the requisite service period of 18 months to four years.

2019 Employee Stock Purchase Plan

Employees may elect to participate in The Magenta Therapeutics, Inc. 2019 Employee Stock Purchase Plan (the "ESPP"). The purchase price of common stock under the ESPP is equal to 85% of the lower of the fair market value of the common stock on the offering date or the exercise date. The six-month offering periods begin in December and June of each year. During the nine months ended September 30, 2022, 49,704 shares of common stock were purchased under the ESPP at a purchase price per share of $0.99. During the nine months ended September 30, 2021, 13,989 shares of common stock were purchased under the ESPP at a purchase price per share of $6.15. As of September 30, 2022, 663,548 shares remained available for issuance under the ESPP.

Stock-Based Compensation

Stock-based compensation expense was classified in the statements of operations and comprehensive loss as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Research and development expenses	$ 315	$ 1,335	$ 1,304	$ 3,276
General and administrative expenses	1,192	2,464	3,953	4,849
	$ 1,507	$ 3,799	$ 5,257	$ 8,125

As of September 30, 2022, unrecognized compensation expense related to unvested share-based awards with service-based vesting conditions was $15.5 million, which is expected to be recognized over a weighted average period of 2.4 years. Additionally, the Company had unrecognized compensation cost of $1.7 million related to the unvested performance restricted stock units for which the performance conditions were not considered probable of achievement as of September 30, 2022.

6. Leases

The Company has a sublease, as amended, for up to approximately 69,000 square feet of office and laboratory space in Cambridge, Massachusetts. The sublease is subject and subordinate to a prime lease between the sublandlord and the prime landlord. The term of the sublease commenced in June 2018 and expires in February 2028. The sublandlord has the right to terminate the sublease after five years. The Company classified this sublease as an operating lease under ASC 842. The Company is obligated to pay real estate taxes and other costs related to the premises, including costs of operations and management of the leased premises. To the extent these costs are variable, they were not included in the measurement of the right-of-use asset and lease liability. In

connection with the sublease, as amended, the sublandlord funded $5.2 million in tenant improvements to the leased facility during 2019. The Company is required to maintain a cash balance of $1.8 million to secure a letter of credit associated with the sublease. This amount was classified as noncurrent restricted cash in the consolidated balance sheets at September 30, 2022 and December 31, 2021.

As of December 31, 2021, the Company had long-term deferred rent of $6.4 million related to lease incentives and payment escalations. As of December 31, 2021, the short-term portion of deferred rent of $0.6 million was included in accrued expenses and other current liabilities. In connection with the adoption of ASC 842 on January 1, 2022, these amounts were recorded as a reduction to the operating lease, right-of-use asset.

The components of the Company's lease expense under ASC 842 were as follows (in thousands):

	Three Months Ended September 30, 2022	Nine Months Ended September 30, 2022
Operating lease cost	$ 1,602	$ 4,805
Short-term lease cost	—	—
Variable lease cost	441	951
	$ 2,043	$ 5,756

Supplemental disclosure of cash flow information related to the lease was as follows (in thousands):

	Three Months Ended September 30, 2022	Nine Months Ended September 30, 2022
Cash paid for amounts included in the measurement of operating lease liabilities	$ 1,674	$ 4,891
Operating lease liabilities arising from obtaining right-of-use asset	$ —	$ —

The weighted average remaining lease term and discount rate were as follows:

	September 30, 2022
Weighted-average remaining lease term - operating lease (in years)	5.42
Weighted-average discount rate - operating lease	11.00%

Because the interest rate implicit in the lease was not readily determinable, the Company's estimated incremental borrowing rate was used to calculate the present value of the lease.

As of September 30, 2022, the future minimum lease payments due under the noncancelable operating lease was as follows (in thousands):

2022 (three months)	$ 1,675
2023	6,936
2024	7,313
2025	7,679
2026	8,062
Thereafter	9,905
Total future minimum lease payments	41,570
Less: imputed interest	(10,772)
Total operating lease liabilities	$ 30,798

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The following table represents the lease liabilities on the consolidated balance sheet (in thousands):

	September 30, 2022
Current operating lease liability	$ 3,621
Operating lease liability, net of current portion	27,177
Total operating lease liabilities	$ 30,798

As previously disclosed in the Company's Annual Report on Form 10-K and under the previous lease accounting standard, ASC 840, *Leases,* the following table summarizes the future minimum lease payments due under the operating lease as of December 31, 2021 (in thousands):

2022	$ 6,375
2023	6,734
2024	7,100
2025	7,455
2026	7,828
Thereafter	9,617
	$ 45,109

Rent expense for the three and nine months ended September 30, 2021 was $1.5 million and $4.6 million, respectively.

In 2018, the Company entered into two sub-subleases of approximately 27,000 square feet of office space in Cambridge, Massachusetts. One of the sub-subleases, as amended, expired in December 2021. The remaining sub-sublease, as amended, was set to expire in April 2022 but was further amended in January 2022 to increase the square footage from 13,643 square feet to 26,114 square feet and to extend the expiration to April 2023. As of September 30, 2022, the remaining base rent payments due to the Company under the amended sub-sublease was $1.5 million. The Company recorded other income of $0.8 million during each of the three months ended September 30, 2022 and 2021, respectively, related to these sub-subleases. The Company recorded other income of $2.2 million and $2.7 million during the nine months ended September 30, 2022 and 2021, respectively, related to these sub-subleases.

7. Commitments and Contingencies

Leases

The Company's commitments under its leases are described in Note 6.

Collaboration Agreement

In March 2018, the Company entered into a collaboration agreement with Heidelberg Pharma Research GmbH ("HDPR") whereby the parties agreed to combine the Company's stem cell platform with proprietary antibodies across up to four exclusive targets with HDPR's proprietary Antibody Targeted Amanitin Conjugates platform. Under the agreement, the Company may pay upfront technology access fees, research exclusivity fees and payment for research support. Additionally, upon the exercise of certain license rights, the Company may be obligated to pay HDPR development, regulatory and commercial milestone payments of up to $83.5 million per target as well as royalties on net sales of products licensed under the agreement. During the three months ended September 30, 2022, the Company did not incur any research and development expenses related to this agreement. During the three months ended September 30, 2021, the Company recorded less than $0.1 million of research and development expense related to this agreement for upfront technology access fees, research exclusivity fees and research support. During each of the nine months ended September 30, 2022 and 2021, the Company recorded $0.4 million of research and development expense related to this agreement for upfront technology access fees, research exclusivity fees and research support. During the nine months ended September 30, 2022, the Company recorded $2.0 million of research and development expense related to the achievement of a development milestone. During the three and nine months ended September 30, 2021, the Company did not incur any expense related to the achievement of these milestones.

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Intellectual Property Licenses

The Company has a license agreement with the President and Fellows of Harvard College ("Harvard"), entered into in November 2016, for an exclusive, worldwide, royalty-bearing license for certain technologies related to conditioning and mobilization. The Company is obligated to pay Harvard maintenance fees of $0.1 million annually and to reimburse qualified expenses related to the patents. The Company is also obligated to pay milestone payments of up to $7.4 million for the first two licensed products upon the achievement of certain development and regulatory milestones and to pay royalties on a product-by-product and country-by-country basis on net sales of products licensed under the agreement. During the three months ended September 30, 2022 and 2021 and the nine months ended September 30, 2022, the Company did not incur any expense related to the achievement of these milestones. During the nine months ended September 30, 2021, the Company recorded $0.1 million of expense related to the achievement of one of these milestones.

The Company has agreements with third parties in the normal course of business, under which it can license certain developed technologies. If the Company exercises its rights to license the respective technologies, it may be subject to additional fees and milestone payments. During the nine months ended September 30, 2022, the Company recorded research and development expense of $0.1 million related to the license of certain developed technologies under these agreements. During the three months ended September 30, 2022 and 2021 and the nine months ended September 30, 2021, the Company did not incur any expense related to these licenses.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors and senior management that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnifications. The Company is not aware of any claims under indemnification arrangements, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of September 30, 2022.

Legal Proceedings

The Company is not currently a party to any material legal proceedings. At each reporting date, the Company evaluates whether or not a potential loss amount or a potential range of loss is probable and reasonably estimable under the provisions of the authoritative guidance that addresses accounting for contingencies. The Company expenses the costs related to its legal proceedings as they are incurred.

8. 401(k) Savings Plan

The Company has a 401(k) available for participating employees who meet certain eligibility requirements. Eligible employees may defer a portion of their salary as defined by the plan. Company contributions to the plan may be made at the discretion of the board of directors of the Company. Effective August 2021, the Company began making matching contributions of up to 2% of eligible wages. During the three and nine months ended September 30, 2022, the Company recorded $0.1 million and $0.2 million of expense, respectively, related to this matching contribution. During the three and nine months ended September 30, 2021, the Company recorded less than $0.1 million of expense related to this matching contribution.

9. Related Parties

Effective March 2018, Amy Lynn Ronneberg, the then serving President of Be The Match BioTherapies, LLC, became a member of the Company's board of directors and subsequently was appointed Chief Executive Officer of the National Marrow Donor Program/Be The Match, or NMDP/Be The Match, organization in June 2020. The Company has collaboration agreements with the National Marrow Donor Program (as successor in interest to Be The Match BioTherapies Collection Services, LLC (formerly known as Be The Match BioTherapies, LLC)) and a research agreement with an affiliated organization, Center for International Blood and Marrow Transplant Research. In addition, in June 2020, the Company entered into a clinical collaboration agreement with NMDP/Be

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The Match to evaluate the potential utility of MGTA-145 for mobilizing and collecting hematopoietic stem cells from donors in a single day and then using them for allogeneic transplant in patients. Under the terms of this agreement, the Company shall fund up to fifty percent of NMDP/Be The Match clinical trial costs and provide the trial drugs which will be included in research and development expense.

During the three months ended September 30, 2022 and 2021, the Company recorded expense of $0.1 million and $0.2 million, respectively, related to these agreements. During the nine months ended September 30, 2022 and 2021, the Company recorded expense of $0.2 million and $0.5 million, respectively, related to these agreements. As of September 30, 2022 and December 31, 2021, amounts on the consolidated balance sheets related to these agreements were less than $0.1 million and $0.2 million, respectively, which amounts were included in accounts payable and accrued expenses and other current liabilities and less than $0.1 million which amounts were included in prepaid expenses and other current assets.

ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS.

FORWARD LOOKING STATEMENTS

This Quarterly Report on Form 10-Q of Magenta Therapeutics, Inc. (the "Company") contains or incorporates statements that constitute forward-looking statements within the meaning of the federal securities laws. Any express or implied statements that do not relate to historical or current facts or matters are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as "may," "will," "could," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "projects," "seeks," "endeavor," "potential," "continue" or the negative of these terms or other comparable terminology. Forward-looking statements appear in a number of places in this Quarterly Report on Form 10-Q and include, but are not limited to, statements about:

- our expectation that our existing capital resources will be sufficient to enable us to fund our currently planned development of MGTA-117, MGTA-145 and our other product candidates;
- the anticipated benefits of our revised operating plan and our expectation that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2024;
- the initiation, timing and success of clinical trials of MGTA-117, MGTA-145 and any other product candidates;
- our ability to commence and enroll patients in our clinical trials at the pace that we project;
- regulatory actions with respect to our product candidates or our competitors' products and product candidates;
- the outcomes of our preclinical studies;
- our ability to manufacture MGTA-117, MGTA-145 or any other product candidate in conformity with the FDA's requirements and to scale up manufacturing of our product candidates to commercial scale, if approved;
- whether the results of our trials will be sufficient to support domestic or foreign regulatory approvals for MGTA-117, MGTA-145 or any other product candidates we may develop;
- our reliance on third parties to conduct our clinical trials;
- our reliance on third-party contract development and manufacturer organizations to manufacture and supply our product candidates for us;
- our ability to establish clinical programs moving forward in multiple indications, with a rapidly advancing portfolio and sustainable platform;
- our ability to obtain, including on an expedited basis, and maintain regulatory approval of MGTA-117, MGTA-145 or any other product candidates we may develop;
- the level of expenses related to any of our product candidates or clinical development programs;
- the benefits of the use of MGTA-117, MGTA-145 or any other product candidate, if approved;
- our ability to successfully commercialize MGTA-117, MGTA-145 or any other product candidates we may identify and pursue, if approved;
- the rate and degree of market acceptance of MGTA-117, MGTA-145 or any other product candidates we may identify and pursue;
- our expectations regarding government and third-party payor coverage and reimbursement;
- our ability to obtain and maintain intellectual property protection for MGTA-117, MGTA-145 or any other product candidates we may identify and pursue;
- our ability to obtain orphan drug designation for any of our product candidates we may identify and pursue;
- our ability to successfully build a specialty sales force and commercial infrastructure;
- our ability to compete with companies currently producing or engaged in the clinical development of treatments for the disease indications that we pursue and treatment modalities that we develop;
- our ability to successfully find collaborators for any of our current and future programs and product candidates;
- our ability to retain and recruit key personnel;

- our estimates of our expenses, ongoing losses, future revenue, capital requirements and our needs for or ability to obtain additional financing;
- our expectations regarding the time during which we will continue to be an emerging growth company or smaller reporting company as defined in federal securities regulations;
- our financial performance; and
- developments and projections relating to our competitors or our industry.

Although we believe that the expectations reflected in these forward-looking statements are reasonable, these statements relate to our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, objectives of management and expected market growth, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from any future results, levels of activity, performance or achievements expressed or implied by these forward-looking statements. You are urged to carefully review the disclosures we make concerning these risks and other factors that may affect our business and operating results under "Item 1A. Risk Factors" in this Quarterly Report on Form 10-Q, as well as our other reports filed with the Securities and Exchange Commission, or the SEC, which disclosures are incorporated herein by reference. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this document. The Company does not intend, and undertakes no obligation, to update any forward-looking information to reflect events or circumstances after the date of this document or to reflect the occurrence of unanticipated events, unless required by law to do so.

Overview

Magenta Therapeutics, Inc. is a clinical-stage biotechnology company developing novel medicines designed to bring the curative power of stem cell transplant to more patients with blood cancers, genetic diseases and autoimmune diseases.

Magenta's drug development pipeline includes multiple clinical and preclinical product candidates designed to improve stem cell transplant. We are developing product candidates that are designed to deplete targeted cells in the bone marrow to make space for the bone marrow to receive newly transplanted stem cells, a process known as conditioning. Our targeted conditioning programs are intended to enhance the efficacy of and/or reduce the dosing levels, intensity or, in some cases, even the need for chemotoxic agents. Our first targeted conditioning program, MGTA-117, has entered clinical development in a Phase 1/2 trial, and our second program, a CD45-antibody drug conjugate, or CD45-ADC, is advancing in preclinical development. In addition to our conditioning programs, we are also developing a product candidate, MGTA-145, to improve the process by which stem cells are stimulated out of the bone marrow and into the bloodstream so they are available for collection for future reinfusion, known as mobilization, which is required for all transplants and gene therapy applications. MGTA-145 is a Phase 2 clinical stage program intended to enable rapid, reliable, predictable and safe mobilization and collection of high numbers of functional stem cells for transplant.

In addition to our product candidates, Magenta's research efforts are evaluating several early-stage targets that include targeted lymphodepletion prior to therapies such as chimeric antigen receptor T-cells or CAR-T.

Magenta intends to become a fully integrated discovery, development, and commercial company in the field of stem cell transplant. We are developing our product candidates to be used individually or, in some cases, in combination with each other or together with other therapies. As a result, our portfolio could be tailored to the patient's disease, such that a patient may receive more than one Magenta therapy as part of his or her individual stem cell transplant.

COVID-19 continues to present operational and other challenges which could delay or halt the development of our product candidates. See "Item 1A. Risk Factors" for further discussion of the current and expected impact on our business and product candidates.

Since our inception in 2015, we have focused substantially all of our efforts and financial resources on organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying potential product candidates and undertaking preclinical studies and clinical trials, including MGTA-117, CD45-ADC and MGTA-145. We do not have any products approved for sale and have not generated any revenue from product sales.

Since our inception, we have incurred significant operating losses. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of our product candidates. Net losses were $56.3 million for the nine months ended September 30, 2022 and $71.1 million for the year ended December 31, 2021. As of September 30, 2022, we had an accumulated deficit of $381.9 million. We expect to continue to incur

significant expenses and increasing operating losses for at least the next several years. We expect our expenses and capital requirements will increase in connection with our ongoing activities, particularly as we:

- continue to enroll and conduct a Phase 1/2 clinical trial for MGTA-117 and Phase 2 clinical trial for MGTA-145;
- initiate and conduct preclinical studies and clinical trials of our other product candidates, including CD45-ADC;
- develop any other future product candidates we may choose to pursue;
- seek marketing approval for any of our product candidates that successfully complete clinical development, if any;
- maintain compliance with applicable regulatory requirements;
- develop and scale up our capabilities to support our ongoing preclinical activities and clinical trials for our product candidates and commercialization of any of our product candidates for which we obtain marketing approval, if any;
- maintain, expand, protect and enforce our intellectual property portfolio;
- develop and expand our sales, marketing and distribution capabilities for our product candidates for which we obtain marketing approval, if any; and
- expand our operational, financial and management systems and increase personnel, including to support our clinical development and commercialization efforts and our operations as a public company.

We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our product candidates. If we obtain regulatory approval for any of our product candidates, we expect to incur significant expenses related to developing our commercialization capability to support product sales, marketing and distribution. Further, we expect to incur additional costs associated with operating as a public company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing and distribution or licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. Additionally, because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. Accordingly, if we fail to raise capital or enter into necessary strategic agreements, or fail to ever become profitable, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates, and we may also be forced to reduce or terminate our operations.

As of September 30, 2022, we had cash, cash equivalents and marketable securities of $128.3 million. Based on our current operating plan, we believe that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2024. See “Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations – Liquidity and Capital Resources.”

Recent Developments

On April 14, 2022, we announced our plan to more narrowly focus our capital allocation on the MGTA-117 targeted conditioning program, the CD45-ADC IND-enabling activities and the MGTA-145 stem cell mobilization efforts in sickle cell disease while also de-prioritizing other portfolio investments. We made certain reductions in our planned spending related to research platform-related investments in new disease targets, paused certain MGTA-145 investments, including the program’s planned MGTA-145 dosing and administration optimization clinical trial in healthy subjects and reduced planned general and administrative expenses. In connection with these reductions to our planned spending, we also reduced our workforce by 14%. Our revised operating plan allows us to extend our cash runway into the second quarter of 2024.

Impact of the COVID-19 Pandemic

The COVID-19 pandemic, including the emergence of various variants, has caused and could continue to cause significant disruptions to the U.S., regional and global economies and has contributed to significant volatility and negative pressure in financial markets.

We have been carefully monitoring the COVID-19 pandemic and its potential impact on our business and have taken important steps to help ensure the safety of our employees and their families and to reduce the spread of COVID-19 in the Cambridge

community. We have established a hybrid work-from-home policy for all employees, as well as safety measures for those using our offices and laboratory facilities that are designed to comply with applicable federal, state and local guidelines instituted in response to the COVID-19 pandemic. We will continue to assess those measures as COVID-19-related guidelines evolve. We have also maintained efficient communication with our partners and clinical sites as the COVID-19 pandemic has progressed. We have taken these precautionary steps while maintaining business continuity so that we can continue to progress our programs.

The future impact of the COVID-19 pandemic on our industry, the healthcare system and our current and future operations and financial condition will depend on future developments, which are uncertain and cannot be predicted with confidence. These developments may include, without limitation, changes in the scope, severity and duration of the pandemic, the actions taken to contain the pandemic or mitigate its impact, including the adoption, administration and effectiveness of available COVID-19 vaccines, the effect of any relaxation of current restrictions within the Cambridge community or regions in which our partners and clinical sites are located and the direct and indirect economic effects of the pandemic and containment measures. See "Item 1A. Risk Factors" for a discussion of the potential adverse impact of COVID-19 on our business, results of operations and financial condition.

Components of Our Results of Operations

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our drug discovery efforts, and the development of our product candidates, which include:

- employee-related expenses, including salaries and related costs, and stock-based compensation expense, for employees engaged in research and development functions;
- expenses incurred in connection with the preclinical and clinical development of our product candidates, including under agreements with contract research organizations, or CROs;
- the cost of consultants and third-party contract development and manufacturing organizations, or CDMOs, that manufacture drug products for use in our preclinical studies and clinical trials;
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and supplies; and
- payments made under third-party licensing agreements.

We expense research and development costs to operations as incurred. Advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Our direct research and development expenses are tracked on a program-by-program basis and consist primarily of external costs, such as fees paid to consultants, central laboratories, contractors, CDMOs and CROs in connection with our preclinical and clinical development activities. We do not allocate employee costs, costs associated with our platform technology or facility expenses, including depreciation or other indirect costs, to specific product development programs because these costs are deployed across multiple product development programs and, as such, are not separately classified.

The successful development and commercialization of our product candidates is highly uncertain. This is due to the numerous risks and uncertainties, including the following:

- successful completion of preclinical studies and clinical trials;
- receipt and related terms of marketing approvals from applicable regulatory authorities;
- raising additional funds necessary to complete clinical development of and commercialize our product candidates;
- obtaining and maintaining patent, trade secret and other intellectual property protection and regulatory exclusivity for our product candidates;
- making arrangements with third-party manufacturers, or establishing manufacturing capabilities, for both clinical and commercial supplies of our product candidates;
- developing and implementing marketing and reimbursement strategies;

- establishing sales, marketing and distribution capabilities and launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- acceptance of our products, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- obtaining and maintaining third-party coverage and adequate reimbursement;
- protecting and enforcing our rights in our intellectual property portfolio;
- maintaining a continued acceptable safety profile of the products following approval; and
- the continuing impact of the COVID-19 pandemic on our industry, the healthcare system, and our current and future operations.

A change in the outcome of any of these variables with respect to the development of any of our product candidates would significantly change the costs and timing associated with the development of that product candidate. We may never succeed in obtaining regulatory approval for any of our product candidates.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect research and development costs to increase significantly for the foreseeable future as our product candidate development programs progress. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. While we do not believe that inflation had a material effect on our financial condition and results of operations during the periods presented, it may result in increased costs in the foreseeable future.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs, and stock-based compensation, for personnel in executive, finance and administrative functions. General and administrative expenses also include direct and allocated facility-related costs and insurance costs, as well as professional fees for legal, patent, consulting, pre-commercialization, accounting and audit services.

Interest and Other Income, Net

Interest and other income, net, consists of interest income and miscellaneous income and expense unrelated to our core operations.

Results of Operations

Comparison of the Three Months Ended September 30, 2022 and 2021

The following table summarizes our results of operations for the three months ended September 30, 2022 and 2021:

	Three Months Ended September 30,		
	2022	2021	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 11,201	$ 10,795	$ 406
General and administrative	6,052	7,450	(1,398)
Total operating expenses	17,253	18,245	(992)
Loss from operations	(17,253)	(18,245)	992
Interest and other income, net	1,190	818	372
Net loss	$ (16,063)	$ (17,427)	$ 1,364

Research and Development Expenses

	Three Months Ended September 30,		
	2022	2021	Change
	(in thousands)		
Direct research and development expenses by program:			
Conditioning	$ 3,943	$ 1,510	$ 2,433
Mobilization	484	973	(489)
Cell therapy	—	42	(42)
Unallocated expenses:			
Personnel-related (including stock-based compensation)	4,189	4,886	(697)
Consultant (including stock-based compensation)	100	544	(444)
Facility related and other	2,485	2,840	(355)
Total research and development expenses	$ 11,201	$ 10,795	$ 406

Expenses related to our conditioning program increased primarily due to an increase of $2.1 million in costs related to CD45-ADC. The increase in costs related to CD45-ADC was primarily due to higher preclinical and manufacturing costs to support our investigational new drug, or IND, enabling studies. The decrease in expenses in our mobilization program was primarily due to a decrease in clinical trial costs related to our Phase 2 investigator-initiated clinical trial in multiple myeloma patients which was completed in the fourth quarter of 2021 and our Phase 2 allogeneic donor clinical trial which was closed in early 2022.

The decrease in personnel-related costs was due primarily to a decrease in stock-based compensation, partially offset by an increase in severance in our research and development function. Personnel-related costs for the three months ended September 30, 2022 and 2021 included stock-based compensation expense of $0.3 million and $1.3 million, respectively. The decrease in consultant costs was due to a decrease in certain research activities as a result of our reprioritization efforts in April 2022. The decrease in facility related and other costs was primarily due to a decrease in lab supplies.

General and Administrative Expenses

	Three Months Ended September 30,		
	2022	2021	Change
	(in thousands)		
Personnel-related (including stock-based compensation)	$ 3,150	$ 4,213	$ (1,063)
Professional and consultant	1,237	1,317	(80)
Facility related and other	1,665	1,920	(255)
Total general and administrative expenses	$ 6,052	$ 7,450	$ (1,398)

The decrease in personnel-related costs was due primarily to a decrease in stock-based compensation. Personnel-related costs for the three months ended September 30, 2022 and 2021 included stock-based compensation expense of $1.2 million and $2.5 million, respectively.

Interest and Other Income, Net

Interest income and other income, net for the three months ended September 30, 2022 consisted primarily of sublease income of $0.8 million and interest income of $0.4 million. Interest income and other income, net for the three months ended September 30, 2021 consisted primarily of sublease income of $0.8 million and interest income of less than $0.1 million.

Comparison of the Nine Months Ended September 30, 2022 and 2021

The following table summarizes our results of operations for the nine months ended September 30, 2022 and 2021:

	Nine Months Ended September 30,		
	2022	2021	Change
		(in thousands)	
Operating expenses:			
Research and development	$ 39,351	$ 33,652	$ 5,699
General and administrative	19,819	20,900	(1,081)
Total operating expenses	59,170	54,552	4,618
Loss from operations	(59,170)	(54,552)	(4,618)
Interest and other income, net	2,886	2,708	178
Net loss	$ (56,284)	$ (51,844)	$ (4,440)

Research and Development Expenses

	Nine Months Ended September 30,		
	2022	2021	Change
		(in thousands)	
Direct research and development expenses by program:			
Conditioning	$ 14,321	$ 6,028	$ 8,293
Mobilization	2,517	3,265	(748)
Cell therapy	31	676	(645)
Unallocated expenses:			
Personnel-related (including stock-based compensation)	14,609	14,193	416
Consultant (including stock-based compensation)	505	1,001	(496)
Facility related and other	7,368	8,489	(1,121)
Total research and development expenses	$ 39,351	$ 33,652	$ 5,699

Expenses related to our conditioning program increased primarily due to an increase of $5.3 million in costs related to CD45-ADC and an increase of $3.3 million in costs related to MGTA-117. The increase in costs related to CD45-ADC was primarily due to higher preclinical and manufacturing costs to support our investigational new drug, or IND, enabling studies. The increase in costs related to MGTA-117 was primarily due to costs incurred upon the achievement of a development milestone under our collaboration agreement and increased costs to support our Phase 1/2 clinical trial which was initiated in December 2021. The decrease in expenses in our mobilization program was primarily due to a decrease in clinical trial costs related to our Phase 2 investigator-initiated clinical trial in multiple myeloma patients which was completed in the fourth quarter of 2021 and our Phase 2 allogeneic donor clinical trial which was closed in early 2022. Expenses related to our cell therapy program decreased as result of the discontinuance of our MGTA-456 program.

The increase in personnel-related costs was due primarily to an increase in severance resulting from our headcount reductions, partially offset by a decrease in stock-based compensation. Personnel-related costs for the nine months ended September 30, 2022 and 2021 included stock-based compensation expense of $1.3 million and $3.2 million, respectively. The decrease in consultant costs was due to a decrease in certain research activities as a result of our reprioritization efforts in April 2022. The decrease in facility related and other costs was primarily due to lower operating costs related to our Cambridge, Massachusetts facility.

General and Administrative Expenses

	Nine Months Ended September 30,		
	2022	2021	Change
		(in thousands)	
Personnel-related (including stock-based compensation)	$ 10,202	$ 10,508	$ (306)
Professional and consultant	3,971	4,785	(814)
Facility related and other	5,646	5,607	39
Total general and administrative expenses	$ 19,819	$ 20,900	$ (1,081)

The decrease in personnel-related costs was due primarily to a decrease in stock-based compensation, partially offset by an increase in headcount. Personnel-related costs for the nine months ended September 30, 2022 and 2021 included stock-based compensation expense of $4.0 million and $4.8 million, respectively. The decrease in professional and consultant costs was primarily due to lower legal, patent and investor relations costs, partially offset by higher recruitment costs.

Interest and Other Income, Net

Interest income and other income, net for the nine months ended September 30, 2022 consisted primarily of sublease income of $2.2 million and interest income of $0.6 million. Interest income and other income, net for the nine months ended September 30, 2021 consisted primarily of sublease income of $2.7 million and interest income of $0.1 million. The decrease in sublease income was due to the expiration of one of our two subleases in December 2021. The increase in interest income was due to higher interest rates.

Liquidity and Capital Resources

Since our inception, we have incurred significant operating losses. We have not yet commercialized any of our product candidates and we do not expect to generate revenue from sales of any product candidates for several years, if at all. Since our initial public offering in June 2018, we have funded our operations primarily with proceeds from the sale of our common stock in both private and public offerings.

We have a shelf registration statement on Form S-3 (the "Shelf") on file with the SEC, which covers the offering, issuance and sale of up to an aggregate of $250.0 million of common stock, preferred stock, debt securities, warrants and/or units of any combination thereof. We also entered into a sales agreement, as amended, with Cowen and Company, LLC, as sales agent to provide for the issuance and sale by us of up to $50.0 million of common stock from time to time in "at-the-market" offerings under the Shelf (the "ATM Program"). The Shelf was declared effective by the SEC on August 12, 2022. During the nine months ended September 30, 2022, we sold 1,644,200 shares of our common stock under the ATM Program at a weighted average price per share of $1.82 resulting in net proceeds of $2.8 million after commissions and offering costs. As of September 30, 2022, $247.0 million remained available under the Shelf, including up to $47.0 million available for sale under the ATM Program.

Cash Flows

The following table summarizes our sources and uses of cash for each of the periods presented:

	Nine Months Ended September 30,	
	2022	2021
	(in thousands)	
Net cash used in operating activities	$ (50,402)	$ (43,841)
Net cash provided by (used in) investing activities	(25,032)	53,751
Net cash provided by financing activities	2,812	89,477
Net increase (decrease) in cash, cash equivalents and restricted cash	$ (72,622)	$ 99,387

Operating Activities

During the nine months ended September 30, 2022, operating activities used $50.4 million of cash, primarily resulting from our net loss of $56.3 million and net cash used by changes in our operating assets and liabilities of $3.3 million, partially offset by non-cash charges of $9.2 million. Net cash used by changes in our operating assets and liabilities for the nine months ended September 30,

2022 consisted primarily of a decrease of $1.0 million in accounts payable and accrued expenses and other current liabilities and a decrease of $2.2 million in operating lease liabilities.

During the nine months ended September 30, 2021, operating activities used $43.8 million of cash, primarily resulting from our net loss of $51.8 million and net cash used by changes in our operating assets and liabilities of $2.3 million, partially offset by non-cash charges of $10.3 million. Net cash used by changes in our operating assets and liabilities for the nine months ended September 30, 2021 consisted primarily of a decrease of $1.3 million in accounts payable and accrued expenses and other current liabilities and an increase of $1.2 million in prepaid expenses and other current assets.

Changes in accounts payable, accrued expenses and other current liabilities and prepaid expenses and other current assets in both periods were generally due to the timing of vendor invoicing and payments.

Investing Activities

During the nine months ended September 30, 2022, net cash used by investing activities was primarily attributable to net purchases of marketable securities of $24.7 million.

During the nine months ended September 30, 2021, net cash provided by investing activities was primarily attributable to maturities of marketable securities of $55.0 million.

Financing Activities

During the nine months ended September 30, 2022, net cash provided by financing activities was $2.8 million, consisting primarily of net proceeds from the issuance of common stock under our ATM Program.

During the nine months ended September 30, 2021, net cash provided by financing activities was $89.5 million, consisting of proceeds from the private placement, net of offering costs, of $86.1 million and proceeds from the exercise of stock options of $3.3 million.

Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities and clinical trials for our product candidates in development. As of September 30, 2022, we had cash, cash equivalents and marketable securities of $128.3 million. We believe that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2024. We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical product candidates, we are unable to estimate the exact amount of our working capital requirements. Our future funding requirements will depend on and could increase significantly as a result of many factors, including those listed above.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our operations through a combination of equity offerings, including sales under our ATM Program, debt financings, collaborations, strategic alliances, marketing and distribution arrangements, or licensing arrangements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights as a common 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 acquisitions or capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances, marketing and distribution arrangements, 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 or other arrangements when needed, we may be required to delay, limit, reduce or terminate our research, 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. Although we continue to pursue these plans, there is no assurance that we will be successful in obtaining sufficient funding on terms acceptable to us to fund our continuing operations, if at all.

Contractual Obligations and Commitments

During the nine months ended September 30, 2022, there were no material changes to our contractual obligations and commitments described in our Annual Report on Form 10-K for the year ended December 31, 2021, as filed with the SEC.

Recently Issued and Adopted Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our consolidated financial statements included in this Quarterly Report on Form 10-Q.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our financial statements and related disclosures requires us to make estimates, assumptions and judgments that affect the reported amount of assets, liabilities, revenue, costs and expenses, and related disclosures. We believe that of our critical accounting policies described under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies and Significant Judgments and Estimates" in our Annual Report on Form 10-K for the year ended December 31, 2021, on file with the SEC, the following involve the most judgment and complexity:

- accrued research and development expenses; and
- stock-based compensation.

Accordingly, we believe the policies set forth above are critical to fully understanding and evaluating our financial condition and results of operations. If actual results or events differ materially from the estimates, judgments and assumptions used by us in applying these policies, our reported financial condition and results of operations could be materially affected.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISKS.

We are a smaller reporting company, as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended, for this reporting period and are not required to provide the information required under this item.

ITEM 4. CONTROLS AND PROCEDURES.

Evaluation of Disclosure Controls and Procedures

Our management, under the supervision and with the participation of our Principal Executive Officer (our President and Chief Executive Officer) and Principal Financial Officer (our Chief Financial and Operating Officer), has evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2022. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Management recognizes that any disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2022, our Principal Executive Officer and Principal Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended September 30, 2022 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS.

From time to time, we may be subject to legal proceedings and claims in the ordinary course of business. We are not currently aware of any such proceedings or claims that we believe will have, individually or in the aggregate, a material adverse effect on our business, financial condition or results of operations.

ITEM 1A. RISK FACTORS.

Set forth below are the risks that we believe are material to our investors and they should be carefully considered. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The risks described below are not intended to be exhaustive and other factors not presently known to us or that we currently believe are immaterial may affect our business, prospects, financial condition and results of operations if they occur. This section contains forward-looking statements. You should refer to the explanation of the qualifications and limitations on forward-looking statements in this Quarterly Report on Form 10-Q.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.

We are a clinical-stage biotechnology company developing novel medicines to bring the curative power of stem cell transplant to more patients and have a limited operating history. Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We have no products approved for commercial sale and have not generated any revenue from product sales to date, and we continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception in June 2015. For the nine months ended September 30, 2022, we reported a net loss of $56.3 million. For the years ended December 31, 2021 and 2020, we reported net losses of $71.1 million and $74.9 million, respectively. As of September 30, 2022, we had an accumulated deficit of $381.9 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for, our product candidates.

Even if we succeed in commercializing one or more of our product candidates, we will continue to incur substantial research and development costs and other expenditures to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We will require additional capital to fund our operations, and if we fail to obtain necessary financing, we will not be able to complete the development and commercialization of our product candidates.

Our operations have consumed substantial amounts of cash since our inception. We expect to continue to spend substantial amounts of cash (including the net proceeds from our initial public offering, or IPO, and our subsequent public and private equity offerings) to conduct further research and development and preclinical testing and clinical trials of our product candidates, to seek regulatory approvals for our product candidates and to launch and commercialize any product candidates for which we receive regulatory approval, including potentially building our own commercial organization to address the U.S., the European Union and certain other markets. As of September 30, 2022, we had approximately $128.3 million in cash, cash equivalents and marketable securities. Our monthly spending levels will vary based on new and ongoing development and corporate activities. Because the length of time and activities associated with successful development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Our future expenses and future funding requirements, both near and long-term, will depend on many factors, including but not limited to:

- the initiation, progress, timing, costs and results of research, preclinical studies and clinical trials for our product candidates;
- the costs to develop, maintain, and enhance a sustainable, scalable, reproducible and transferable manufacturing process for our product candidates;
- the clinical development plans we establish for these product candidates;

- the number and characteristics of product candidates that we develop or may in-license;
- the cost of milestone or other payments under any current or future license, acquisition, collaboration or other strategic transaction agreements;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA, the EMA and other comparable foreign regulatory authorities;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending material intellectual property disputes, including patent infringement actions brought by third parties against us or our product candidates;
- the effect of competing technological and market developments;
- the cost and timing of completion of commercial-scale outsourced manufacturing activities;
- the cost of seeking to attract, hire and retain skilled personnel;
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own; and
- the cost of, and ability to maintain on reasonable commercial and economic terms, sufficient office and laboratory space to support our operations.

We cannot be certain that additional funding will be available on acceptable terms, or at all, and such funding may become even more difficult to obtain due to rising interest rates and the current downturn in the U.S. capital markets and the biotechnology sector in general. Competition for additional capital among biotechnology companies may be particularly intense during this present economic downturn. We may be unable to raise capital through public offerings of our common stock and may need to turn to alternative financing arrangements. Such arrangements, if we pursue them, could involve issuances of one or more types of securities, including common stock, preferred stock, convertible debt, warrants to acquire common stock or other securities. These securities could be issued at or below the then prevailing market price for our common stock. In addition, if we issue debt securities, the holders of the debt would have a claim to our assets that would be superior to the rights of stockholders until the principal, accrued and unpaid interest and any premium or make-whole has been paid. Interest on any newly-issued debt securities and/or newly-incurred borrowings would increase our operating costs and reduce our net income, and these impacts may be material. If the issuance of new securities results in diminished rights to holders of our common stock, the market price of our common stock could be materially and adversely affected. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates or one or more of our other research and development initiatives, and we may also be forced to reduce or terminate our operations.

We also could be required to 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. We may also be required to relinquish or license on unfavorable terms our rights to technologies or product candidates that we otherwise would seek to develop or commercialize ourselves. As a result, we may fail to realize the full potential of our product candidates.

Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

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

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates or grant licenses on terms unfavorable to us.

Our company has a limited operating history and no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for our future viability.

We are a clinical-stage company. We were founded and commenced operations in June 2015. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying potential product candidates, and undertaking preclinical studies and clinical trials. Although we have initiated clinical trials for certain of our product candidates, we have not yet demonstrated an ability to successfully complete certain clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial-scale medicine or arrange for a third party to do so on our behalf or conduct sales and marketing activities necessary for successful commercialization. Typically, it takes about 10 to 15 years to develop a new medicine from the time it is discovered to when it is available for treating patients. Consequently, any predictions we make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as our business grows, we may encounter unforeseen expenses, difficulties, complications, delays, and other known and unknown factors. We will need to transition at some point from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition. For example, management may fail to undertake sufficient risk mitigation strategies for elements of our business subject to heightened risk, and as a result our business may be harmed.

We have never generated revenue from product sales and may never be profitable.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, product candidates we may identify for development. We may not generate revenues from product sales for the next several years, if ever. Our ability to generate future revenues from product sales depends heavily on our and or our collaborators' ability to successfully:

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

Even if one or more of the product candidates we may develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, the EMA, or other regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

Risks Related to Product Development and Regulatory Approval

We are early in our development efforts for our product candidates. If we are unable to advance our product candidates through development, obtain regulatory approval and commercialize them, or if we experience significant delays in doing so, our business will be materially harmed.

We are early in our development efforts for our product candidates, including MGTA-117 and MGTA-145. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development, regulatory approval and commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any product, and we may never be able to develop or commercialize a marketable product.

Each of our programs and product candidates will require additional preclinical and clinical development, regulatory approval, potentially in multiple jurisdictions, obtaining manufacturing supply, capacity and expertise, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenue from product sales. Our product candidates must be authorized for marketing by the FDA, or certain other foreign regulatory agencies, such as the EMA, before we may commercialize our product candidates in the U.S. or other countries.

The success of our product candidates will depend on several factors, including the following:

- successful completion of preclinical studies and successful enrollment and completion of clinical trials, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable, under the FDA's current Good Clinical Practices, or cGCPs, and the FDA's current Good Laboratory Practices, or cGLP;
- effective IND applications or Clinical Trial Authorizations that allow commencement of our planned clinical trials or future clinical trials for our product candidates;
- positive results from our preclinical and clinical programs that support a finding of safety and effectiveness and an acceptable risk-benefit profile of our product candidates in the intended populations;
- receipt of regulatory approvals from applicable regulatory authorities;
- establishment of arrangements with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- successful development of our internal or external manufacturing processes or transfer to larger-scale facilities operated by either a third-party contract development and manufacturing organization, or CDMO, or by us;
- establishment and maintenance of patent and trade secret protection or regulatory exclusivity for our product candidates;
- commercial launch of our product candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of our product candidates, if and when approved, by patients, the medical community and third-party payors;
- effective competition against other therapies, including certain chemotherapies;
- establishment and maintenance of healthcare coverage and adequate reimbursement;
- enforcement and defense of intellectual property rights and claims; and
- maintenance of a continued acceptable safety profile of our product candidates following approval.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

The successful development of biopharmaceuticals and cell-based therapies is highly uncertain.

Successful development of biopharmaceuticals and cell-based therapies is highly uncertain and is dependent on numerous factors, many of which are beyond our control. Blood and immune reset and cell-based therapies that appear promising in the early phases of development may fail to reach the market for several reasons including:

- preclinical study results may show the therapies to be less effective than desired or to have harmful or problematic side effects;

- clinical trial results may show the therapies to be less effective than expected (e.g., the trial failed to meet its primary endpoint or the results are not competitive compared to other therapeutic alternatives) or to have unacceptable side effects or toxicities;
- failure to receive the necessary regulatory approvals or a delay in receiving such approvals, which delays may be caused by, among other things, slow enrollment in clinical trials, delays due to investigations concerning safety, length of time to achieve study endpoints, additional requirements for data by regulatory agencies, additional time requirements for data analysis, or biologics license application, or BLA, new drug application, or NDA, preparation, discussions with the FDA, an FDA request for additional preclinical or clinical data, or unexpected safety or manufacturing issues;
- manufacturing costs, formulation issues, pricing or reimbursement issues, or other factors that make the therapy uneconomical; and
- the proprietary rights of others and their competing products and technologies that may prevent the therapy from being commercialized.

Success in preclinical studies and early clinical trials does not ensure that large-scale clinical trials will be successful. The length of time necessary to complete clinical trials and to submit an application for marketing approval for a final decision by a regulatory authority varies significantly from one therapy to the next and may be difficult to predict.

Even if we are successful in getting market approval, third-party payers could require us to conduct additional studies, including post-marketing studies related to the cost effectiveness of a product, to qualify for reimbursement, which could be costly and divert our resources.

In addition, if one of our product candidates is approved for marketing, we will be subject to significant regulatory obligations regarding the submission of safety and other post-marketing information and reports and registration, and we will need to continue to comply (or ensure that our third-party providers comply) with the FDA's current Good Manufacturing Practices, or cGMP, and cGCP requirements for any clinical trials that we conduct post-approval. In addition, there is always the risk that we or a regulatory authority might identify previously unknown problems with a product post-approval, such as adverse events of unanticipated severity or frequency. Compliance with these requirements is costly, and any failure to comply or other issues with our product candidates' post-market approval could have a material adverse effect on our business, financial condition and results of operations.

Our ongoing and planned clinical trials or those of our collaborators may reveal significant adverse events not seen in our preclinical and clinical studies and may result in a safety profile that could inhibit regulatory approval or market acceptance of any of our product candidates.

Before obtaining regulatory approvals for the commercial sale of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target indication. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. There is typically an extremely high rate of attrition from the failure of product candidates proceeding through clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or unacceptable safety issues, notwithstanding promising results in earlier trials. Most product candidates that commence clinical trials are never approved as products.

It is impossible to predict when or if any product candidates we may develop will prove safe in humans. If any product candidates we develop are associated with serious adverse events, undesirable side effects or have characteristics that are unexpected, we may need to abandon their development or limit development to certain uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations and prospects. It is possible that product candidates that initially showed promise in early-stage testing will later have been found to cause side effects that prevent further clinical development of the product candidates.

If significant adverse events or other side effects are observed in any of our current or future clinical trials, we may have difficulty recruiting patients to the clinical trial, patients may drop out of our trial, or we may be required to abandon the trial or our development efforts of that product candidate altogether. We, the FDA or other applicable regulatory authorities, or an institutional review board, or IRB, may suspend clinical trials of a product candidate at any time for various reasons, including a belief that subjects in such trials are being exposed to unacceptable health risks or adverse side effects. Some potential therapeutics developed in

the biotechnology industry that initially showed therapeutic promise in early-stage studies have later been found to cause side effects that prevented their further development. Even if the side effects do not preclude the drug from obtaining or maintaining marketing approval, undesirable side effects may inhibit market acceptance of the approved product due to its tolerability versus other therapies. Any of these developments could materially harm our business, financial condition and prospects.

Stem cell transplant is a high-risk procedure with curative potential that may result in complications or adverse events for patients in our clinical trials or for patients that use any of our product candidates, if approved.

Stem cell transplant can cure patients across multiple diseases, but its use carries with it risks of toxicity, serious adverse events and death. Because many of our therapies are used to prepare or treat patients undergoing stem cell transplant, patients in our clinical trials or patients that use any of our product candidates may be subject to many of the risks that are currently inherent to this procedure. In particular, stem cell transplant involves certain known potential post-procedure complications that may manifest several weeks or months after a transplant and which may be more common in certain patient populations. If serious adverse events, undesirable side effects, evidence of lower than expected efficacy, or unexpected characteristics are identified during the development of any of our product candidates, we may need to limit, delay or abandon our further clinical development of those product candidates, even if such events, effects or characteristics were the result of stem cell transplant or related procedures generally and were not directly or specifically caused or exacerbated by our product candidates. In the event we need to limit, delay or abandon the clinical development of any of our product candidates, our business will likely be materially adversely affected.

In addition, patients who are in our clinical studies or undergoing stem cell transplant typically have underlying disorders or compromised immune systems that make them vulnerable or fragile for undergoing additional clinical studies. For example, we are enrolling patients for our Phase 1/2 clinical trial of MGTA-117 in patients with acute myeloid leukemia, or AML, and myelodysplastic syndrome, or MDS; however, the patients in this study are subject to underlying disorders that may cause negative outcomes for those patients that could slow down the trial, prevent the trial from moving to the next phase or even suspend the trial. As a result, the FDA could put the trial on clinical hold until we can satisfy any potential FDA concerns. In the event that the FDA places a clinical hold on any of our trials, if any FDA clinical hold is not lifted or if the process takes an extended period of time, our business and prospects may suffer material adverse consequences.

All serious adverse events or unexpected side effects are continually monitored per the clinical trial's approved protocol. If serious adverse events are determined to be directly or specifically caused or exacerbated by our product candidates, we would follow the trial protocol's requirements, which call for our data safety monitoring committee to review all available clinical data in making a recommendation regarding the trial's continuation.

If we are not able to identify a safe and effective dose for any of our product candidates, including our antibody drug conjugates, or ADCs, such as MGTA-117 utilizing an amanitin toxin not previously tested in humans, we may need to delay, abandon or limit our development of any potential product candidates.

We may not be able to identify a safe and effective dose for some of our current or future product candidates, and as a result we may need to delay, abandon or limit our development of our product candidates. Some of our product candidates utilize ADCs, which utilize toxins to kill cells. ADCs, including those that have received marketing approval, have dose-dependent safety findings that can include liver toxicity, depending on the target of the ADC and the drug used in the conjugate. In addition, ADCs may have other adverse side effects including fatalities. Although our CD117-ADC, which was designed to deplete hematopoietic stem cells, or HSCs, was generally well tolerated at efficacious doses in non-human primate studies, we may not be able to ultimately show that MGTA-117 can deplete HSCs at a safe and effective dose in humans, and we may need to delay, abandon or limit these development efforts. The dose required for efficacy may differ for different populations, for example between adult and pediatric populations, between populations with diseases that involve bone marrow to different extents, or between uses of MGTA-117 as a monotherapy or as combination with other therapeutic agents. Additional trials to determine the safe and effective dose for different settings of use of MGTA-117 may be required. Further, MGTA-117 utilizes an amanitin toxin that has not been previously tested as an ADC toxin in humans. Other companies, for example Heidelberg Pharma (whether alone or with third parties), are developing ADCs with amanitin toxins and have begun clinical trials. If such other trials encounter safety or efficacy issues, especially if related to the amanitin toxin, then our MGTA-117 program may be adversely affected, and our business may be materially harmed.

Clinical development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of any product candidates.

Our product candidates are in the preclinical development and clinical trial stages, and their risk of failure is high. It is impossible to predict when or if any of our programs will prove effective and safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical

development and then conduct extensive clinical trials to demonstrate the safety and efficacy of any of our future product candidates in humans. Preclinical and clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. We may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful, and a clinical trial can fail at any stage of testing. The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

Successful completion of clinical trials is a prerequisite to submitting a BLA or NDA to the FDA, a Marketing Authorization Application to the EMA and similar approval filings to comparable foreign regulatory authorities, for each product candidate and, consequently, the ultimate approval and commercial marketing of any product candidates. Our current and planned clinical trials may be delayed or may not be completed on schedule, if at all, and this may lead to delay in obtaining regulatory approval for our product candidates.

We may experience delays in completing our preclinical studies and initiating or completing clinical trials. We also may experience numerous unforeseen events during, or as a result of, any current or future clinical trials that we could conduct that may delay or prevent our ability to develop, receive marketing approval for or commercialize our product candidates, including:

- regulators, IRBs, or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or fail to reach, agreement on acceptable terms with prospective trial sites and prospective contract research organization, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- we may generate insufficient or unfavorable data that may delay or prevent us from expanding our Phase 1/2 clinical trial of MGTA-117 in patients with AML and MDS to include stem cell transplant eligible patients, or the FDA may otherwise delay or prevent us from expanding the trial into this patient population;
- clinical trials of any product candidates may fail to show safety or efficacy, produce negative or inconclusive results and we may decide, or regulators may require us, to conduct additional preclinical studies or clinical trials or we may decide to abandon product development programs;
- the number of patients required for clinical trials of any product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, or participants may drop out of these clinical trials or fail to return for post-treatment follow-up at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or may deviate from the clinical trial protocol or drop out of the trial, which may require that we add new clinical trial sites or investigators;
- we may elect to, or regulators, IRBs or ethics committees may require, that we or our investigators, suspend or terminate clinical research or trials for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of preclinical studies and clinical trials of any product candidates may be greater than we anticipate, and/or greater than we have budgeted for;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, IRBs or ethics committees to suspend or terminate the trials, or reports may arise from preclinical or clinical testing of other blood and immune reset and cell-based therapies that raise safety or efficacy concerns about our product candidates.

We could also encounter delays if a clinical trial is suspended or terminated by us, the IRBs of the institutions in which such trials are being conducted, the Data Safety Monitoring Board for such trial, or the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product or treatment, failure to establish or achieve clinically meaningful trial endpoints, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates. Further, the FDA or other regulatory authorities may disagree with our clinical trial design and our interpretation of data from clinical trials or may change the requirements for approval even after they have reviewed and commented on the design for our clinical trials.

Our product development costs will increase if we experience delays in clinical testing or marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates and may allow our competitors to bring products to market before we do, potentially impairing our ability to successfully commercialize our product candidates and harming our business and results of operations. Any delays in our preclinical or future clinical development programs may harm our business, financial condition and prospects significantly.

In connection with our plan announced on April 14, 2022 to more narrowly focus our capital allocation on the MGTA-117 targeted conditioning program, the CD45-ADC IND-enabling activities and the MGTA-145 stem cell mobilization efforts in sickle cell disease, we de-prioritized other portfolio investments, made certain reductions in our planned spending related to research platform-related investments in new disease targets and paused certain MGTA-145 investments, including the program's planned MGTA-145 dosing and administration optimization clinical trial in healthy subjects. There is an additive degree of risk to any development program that is paused or de-prioritized because the time to restart the program and the associated expense may be longer and more costly than previously anticipated. It may also not be possible to restart the de-prioritized program altogether.

If we encounter delays or difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may experience delays or difficulties in patient enrollment in our clinical trials for a variety of reasons, including impacts that have resulted, or may in the future result, from the COVID-19 pandemic. The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the trial until its conclusion. The enrollment of patients depends on many factors, including:

- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- the proximity of patients to trial sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- our ability to obtain and maintain patient consents;
- the risk that patients enrolled in clinical trials will drop out of the trials before completion; and
- adequate staffing at institutions running our clinical trials to efficiently conduct such trials.

In addition, our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in such clinical trial site.

Delays in patient enrollment may result in increased costs or may affect the timing or outcome of the planned clinical trials, which could prevent completion or advancement of these trials into the next phase and may adversely affect our ability to advance the development of our product candidates. Delays in patient enrollment may also result in delays in obtaining data for our clinical trials, including our Phase 1/2 clinical trial of MGTA-117 in patients with AML and MDS and our Phase 2 clinical trial of MGTA-145 in patients with sickle cell disease, which may adversely affect our ability to engage with regulatory authorities to advance the study and to raise capital.

Results of preclinical studies and early clinical trials may not be predictive of results of future clinical trials, and such results do not guarantee approval of a product candidate by regulatory authorities.

The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of clinical trials do not necessarily predict success in the results of completed clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in earlier development, and we could face similar setbacks. The design of a clinical trial can determine whether its results will support approval of a product, and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. In addition, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain regulatory approval for their product candidates.

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial procedures and the rate of dropout among clinical trial participants. If we fail to receive positive results in clinical trials of our product candidates, the development timeline and regulatory approval and commercialization prospects for our most advanced product candidate, and, correspondingly, our business and financial prospects would be negatively impacted.

Additionally, several of our past, planned and ongoing clinical trials utilize an "open-label" trial design. An "open-label" clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a "patient bias" where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an "investigator bias" where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results with any of our product candidates for which we include an open-label clinical trial when studied in a controlled environment with a placebo or active control.

Even if we complete clinical development of MGTA-117, MGTA-145 or any other product candidates, there can be no assurance that the FDA, EMA, or other regulatory authorities will approve MGTA-117, MGTA-145 or any other product candidates for marketing. For example, in the course of the COVID-19 public health emergency, a number of companies have announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications.

Interim, preliminary and "topline" data from our clinical trials that we announce or publish from time to time may change as more patient data become available following the interim data; preliminary data are subject to audit and verification procedures, and deeper analysis of the data beyond the topline data may provide more color and context to the data, all of which could result in material or other changes in the final data.

From time to time, we may disclose interim data from our preclinical studies and clinical trials, which are based on an interim analysis of then-available data from ongoing studies or trials. Interim data from our preclinical studies and clinical trials that we may complete are subject to the risk that one or more of the clinical observations may materially change as patient enrollment continues and more patient data become available from the particular study or trial. As a result, interim data should be viewed with caution until final data are available. Adverse differences between interim data and final data could significantly harm the development of our product candidates and our business prospects with respect thereto.

We may also announce or publish preliminary data from our preclinical studies or clinical trials that are based on a preliminary analysis of final data. Preliminary data from our preclinical studies and clinical trials are subject to change following a more comprehensive review of the data from the particular preclinical study or trial. We also make assumptions, estimations, calculations and conclusions as part of our preliminary analyses of the data, and we may not have received, or had the opportunity to fully and carefully evaluate, all of the data at the time of making such assumptions, estimations, calculations and/or conclusions. As a result, preliminary data remain subject to audit and verification procedures that may result in the final data being different from the preliminary data we previously announced or published.

We may also announce or publish topline data from our preclinical studies and clinical trials, which are a subset of the total data and are intended to provide the important results from the study or trial. Deeper analysis of the data beyond the topline data may provide more color and context to the results. If the additional color or context shows, in retrospect, that the topline data was

incomplete or adverse, it could significantly harm the development of our product candidate and our business prospects with respect thereto.

Further, third parties, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses, or they may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate and our business prospects. In addition, the information we announce or publish regarding a particular preclinical study or clinical trial may represent only a portion of extensive information generated from that study or trial, and our shareholders or other third parties may not agree with what we determine is material, important or otherwise appropriate information to include in our disclosure.

If the interim, preliminary, or topline data that we report differ materially from final results, or if third parties, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business prospects, operating results or financial condition. Further, announcement of preliminary, interim or topline data by us, or differences between that data and the final data, could result in volatility in the price of our common stock.

We may not be successful in our efforts to identify additional product candidates. Due to our limited resources and access to capital, we must prioritize development of certain product candidates; these decisions may prove to be wrong and may adversely affect our business.

Although we intend to explore other therapeutic opportunities, in addition to the product candidates that we are currently developing, we may fail to identify successful product candidates for clinical development for a number of reasons. If we fail to identify additional potential product candidates, our business could be materially harmed.

Research programs to pursue the development of our planned product candidates for additional indications and to identify new product candidates and disease targets require substantial technical, financial and human resources whether or not they are ultimately successful. Our research programs may initially show promise in identifying potential indications and/or product candidates, yet fail to yield results for clinical development for a number of reasons, including:

- the research methodology used may not be successful in identifying potential indications and/or product candidates;
- potential product candidates may, after further study, be shown to have harmful adverse effects or other characteristics that indicate they are unlikely to be effective drugs; or
- it may take greater human and financial resources than we possess to identify additional therapeutic opportunities for our product candidates or to develop suitable potential product candidates through internal research programs, thereby limiting our ability to develop, diversify and expand our product portfolio.

Because we have limited financial and human resources, we intend to initially focus on research programs and product candidates for a limited set of 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 or a greater likelihood of success. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities.

Accordingly, there can be no assurance that we will ever be able to identify additional therapeutic opportunities for our product candidates or to develop suitable potential product candidates through internal research programs, which could materially adversely affect our future growth and prospects. We may focus our efforts and resources on potential product candidates or other potential programs that ultimately prove to be unsuccessful.

If we are unable to successfully develop our current programs into a comprehensive portfolio of product candidates, or experience significant delays in doing so, we may not realize the full commercial potential of our current and future product candidates.

We are developing our product candidates so that they can each be used individually or in combination with each other. In particular, we are focused on a product development strategy that includes leveraging the synergies among a comprehensive portfolio of our product candidates. Our success may depend, in part, on our ability to develop a complementary product portfolio with product candidates that, together or individually, will address the major opportunities inherent in the existing stem cell transplant process. Given our limited experience in developing product candidates, we may not be successful in developing some of our product candidates. The failure of one of our product candidates to obtain regulatory approval or market acceptance may affect our ability to expand our market opportunities for our other product candidates or programs. Although we may develop product candidates that ultimately obtain marketing approval, if we are unable to successfully develop our current programs into a comprehensive portfolio of

product candidates, or experience significant delays in doing so, we may not realize the full commercial potential of our current and future product candidates.

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

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications among many potential options. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial medicines 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 medicines. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing, or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Any such event could have a material adverse effect on our business, financial condition, results of operations, and prospects.

We have no experience as a company in obtaining regulatory approval for a drug or biologic.

As a company, we have never obtained regulatory approval for, or commercialized, a drug or biologic. It is possible that the FDA may refuse to accept any or all future NDAs or BLAs for substantive review or may conclude after review of our data that our application is insufficient to obtain regulatory approval for any current or future product candidates. If the FDA does not approve any future NDAs or BLAs, it may require that we conduct additional costly clinical, preclinical or manufacturing validation studies before it will reconsider our applications. Depending on the extent of these or any other FDA-required studies, approval of any NDA or BLA or other application that we submit may be significantly delayed, possibly for several years, or may require us to expend more resources than we have available. Any failure or delay in obtaining regulatory approvals would prevent us from commercializing MGTA-117, MGTA-145 or any other product candidate, generating revenues and achieving and sustaining profitability. It is also possible that additional studies, if performed and completed, may not be considered sufficient by the FDA to approve any NDA, BLA or other application that we submit. If any of these outcomes occur, we may be forced to abandon the development of our product candidates, which would materially adversely affect our business and could potentially cause us to cease operations. We face similar risks for our applications in foreign jurisdictions.

Because we are developing product candidates for the treatment of diseases in which there is little clinical experience using new technologies, there is increased risk that the FDA, the EMA, or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results, and these results may be difficult to analyze.

During the regulatory review process, we will need to identify clinical trial designs, success criteria, and endpoints such that the FDA, the EMA, or other regulatory authorities will be able to determine the clinical efficacy and safety profile of any product candidates we may develop. As we are initially seeking to identify and develop product candidates to treat diseases in which there is little clinical experience using new technologies, there is heightened risk that the FDA, the EMA, or other regulatory authorities may not consider the clinical trial design or endpoints that we propose to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. The FDA, the EMA, or other regulatory authorities may lack the specific subject matter knowledge or guiding historical precedent to properly analyze the clinical data and results from our clinical trials, which may adversely affect our ability to obtain regulatory approval for our product candidates.

Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. This may be a particularly significant risk for many of the genetically defined diseases for which we plan to develop product candidates because many of these diseases have small patient populations, and designing and executing a rigorous clinical trial with appropriate statistical power is more difficult than with diseases that have larger patient populations. Further, even if we do achieve the pre-specified criteria, we may produce results that are unpredictable or inconsistent with the results of the non-primary endpoints or other relevant data. The FDA also weighs the benefits of a product against its risks, and the FDA may view the efficacy results in the context of safety as not being supportive of regulatory approval. Other regulatory authorities in the European Union and other countries may make similar comments with respect to these endpoints and data. Any product candidates we may develop will be based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval, if at all.

Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate we may develop, and any such approval may be for a narrower indication than we seek.

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if any product candidates we may develop meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials, and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested, or they may impose significant limitations in the form of narrow indications, warnings or distribution and use restrictions under a Risk Evaluation and Mitigation Strategy, or REMS. These regulatory authorities may require precautions or contra-indications with respect to conditions of use, or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of any product candidates we may develop. Any of the foregoing scenarios could materially harm the commercial prospects for any product candidates we may develop and materially adversely affect our business, financial condition, results of operations and prospects.

Even if we obtain regulatory approval of any of our product candidates, the approved products may be subject to post-approval studies and will remain subject to ongoing regulatory requirements. If we fail to comply, or if concerns are identified in subsequent studies, our approval could be withdrawn, and our product sales could be suspended.

If we are successful in obtaining regulatory approval for MGTA-117, MGTA-145 or any of our other product candidates, regulatory agencies in the U.S. and other countries where a product will be sold may require extensive additional clinical trials or post-approval clinical trials that are expensive and time-consuming to conduct. Alternatively, we may not be able to conduct such additional trials, which might force us to abandon our efforts to develop or commercialize certain product candidates. Even if post-approval studies are not requested or required, after our products are approved and on the market, there might be safety issues that emerge over time that require a change in product labeling, additional post market studies or clinical trials, imposition of distribution and use restrictions under a REMS, or withdrawal of the product from the market, which would cause our revenue to decline.

Additionally, any products that we may successfully develop will be subject to ongoing regulatory requirements after they are approved. These requirements will govern the manufacturing, packaging, marketing, distribution, and use of our products. If we fail to comply with such regulatory requirements, approval for our products may be withdrawn, and product sales may be suspended. We may not be able to regain compliance, or we may only be able to regain compliance after a lengthy delay, significant expense, lost revenues and damage to our reputation.

A Breakthrough Therapy Designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.

We plan to seek a Breakthrough Therapy Designation for our product candidates if the clinical data support such a designation for one or more product candidates.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under non-expedited FDA review procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product no longer meets the conditions for qualification.

The regenerative medicine advanced therapy, or RMAT, designation by the FDA for any of our product candidates may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.

We plan to seek an RMAT designation for our product candidates if the clinical data support such a designation for one or more product candidates.

Designation as an RMAT is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a RMAT, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of RMAT designation for our product candidates may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify for RMAT designation, the FDA may later decide that the biological products no longer meet the conditions for qualification.

Accelerated approval by the FDA, even if granted for our current or any other future product candidates, may not lead to a faster development, regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive regulatory approval.

We may seek accelerated approval of our current or future product candidates using the FDA's accelerated approval pathway. Even if we do receive accelerated approval, however, we may not experience a faster development, regulatory review or approval process, and receiving accelerated approval does not provide assurance of ultimate FDA approval for our product candidates.

Our current product candidates and future product candidates may not be eligible for Orphan Drug status.

The FDA granted Orphan Drug designation to MGTA-145 for the mobilization of HSCs to the peripheral blood for collection and subsequent transplant in May 2020. We plan to seek Orphan Drug designation for our other product candidates if the clinical data support such a designation. The U.S. and Europe may designate drugs for relatively small patient populations as orphan drugs. Orphan Drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process, but does make the product eligible for orphan drug exclusivity, reduced filing fees and specific tax credits. Generally, if a company receives the first marketing approval for a product with an Orphan Drug designation in the clinical indication for which it has such designation, the product is entitled to orphan drug exclusivity. Orphan drug exclusivity means that the FDA will not approve another application to market the same drug for the same indication, except in limited circumstances, for a period of seven years in the U.S. This exclusivity, however, could block the approval of our proposed product candidates if a competitor obtains marketing approval before us. However, even if we obtain orphan drug exclusivity for any of our proposed product candidates, we may not be able to maintain it. For example, if a competitive product is shown to be clinically superior to our product candidates, any orphan drug exclusivity we have will not block the approval of such competitive product.

On August 3, 2017, Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's preexisting regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The law reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. Moreover, in the Consolidated Appropriations Act of 2021, Congress did not further change this interpretation when it clarified that the interpretation codified in FDARA would apply in cases where the FDA issued an orphan designation before the enactment of FDARA but where product approval came after the enactment of FDARA. The FDA may further reevaluate its regulations and policies under the Orphan Drug Act. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

A Fast Track Designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development, regulatory review or approval process and does not increase the likelihood that our product candidates will receive marketing approval.

The FDA has broad discretion whether or not to grant a Fast Track Designation for a particular indication, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Receipt of Fast Track Designation may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not guarantee qualification for the FDA's priority review procedures. In addition, the FDA may withdraw any Fast Track Designation at any time. We may seek Fast Track Designation for our product candidates, but there is no assurance that the FDA will grant this status to any of our proposed product candidates.

We may seek priority review designation for our product candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

We may request priority review for our product candidates, however, we cannot assume that our product candidates will meet the criteria for that designation. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster development or regulatory review or

approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

Inadequate funding for the FDA, the SEC and other government agencies, including from government shutdowns, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and 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. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those 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 product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years the U.S. government has shut down several times and certain regulatory authorities, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical 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. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Separately, since March 2020 when foreign and domestic inspections of facilities were largely placed on hold, the FDA has been working to resume pre-pandemic inspection activities, including routine surveillance, bioresearch monitoring and pre-approval inspections. Should the FDA determine that an inspection is necessary for approval and an inspection cannot be completed during the review cycle due to restrictions on travel, and the FDA does not determine a remote interactive evaluation to be adequate, the agency has stated that it generally intends to issue, depending on the circumstances, a complete response letter or defer action on the application until an inspection can be completed. In the course of the COVID-19 pandemic, a number of companies have announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. Regulatory authorities outside the U.S. may adopt similar restrictions or other policy measures in response to the ongoing COVID-19 pandemic and may experience delays in their regulatory activities. If a prolonged government shutdown or other disruption 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. Future shutdowns or other disruptions could also affect other government agencies, such as the SEC, which may also impact our business by delaying review of our public filings, to the extent such review is necessary, and our ability to access the public markets.

Risks Related to Reliance on Third Parties and Manufacturing

We rely on third parties to conduct our preclinical and clinical trials, process and product development and GMP manufacturing, and we will rely on them to perform other tasks for us as well. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

Although we have recruited a team that has experience with clinical trials, as a company we have relied upon, and plan to continue to rely upon, medical institutions, clinical investigators, contract laboratories, our CROs and other third parties to conduct preclinical studies and future clinical trials for our product candidates. We expect to rely heavily on these parties for execution of preclinical and future clinical trials for our product candidates, and we control only certain aspects of their activities. Nevertheless, we will be responsible for ensuring that each of our preclinical and clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on CROs will not relieve us of our regulatory responsibilities. For any violations of laws and regulations during the conduct of our preclinical studies and clinical trials, we could be subject to warning letters or enforcement action that may include civil penalties up to and including criminal prosecution.

We and our CROs will be required to comply with regulations, including cGCPs and cGLPs for conducting, monitoring, recording and reporting the results of preclinical and clinical trials to ensure that the data and results are scientifically credible and accurate, and that the trial patients are adequately informed of the potential risks of participating in clinical trials and their rights are

protected. These regulations are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for any drugs in clinical development. The FDA enforces cGCP regulations through periodic inspections of clinical trial sponsors, principal investigators and trial sites. If we or our CROs fail to comply with applicable cGCPs or cGLP, 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, the FDA will determine that any of our future clinical trials will comply with cGCPs or cGLPs. In addition, our clinical trials must be conducted with product candidates produced in accordance with the requirements in the FDA's cGMP requirements. Our failure or the failure of our CROs to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process and could also subject us to enforcement action.

Although we intend to design our planned clinical trials for our product candidates, for the foreseeable future CROs will conduct all of our planned clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct future preclinical studies and clinical trials will also result in less day-to-day control over the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, any preclinical studies or clinical trials with which such CROs are associated with may be extended, delayed or terminated. In such cases, we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates in the subject indication could be harmed, our costs could increase and our ability to generate revenue could be delayed.

Furthermore, we may be reliant on cooperation of third parties for access to unique methods or unique therapeutic agents for specific trials. For example, determination of specific endpoints may require specific methods, or development of drugs in combination may require access to specific drugs. Lack of access or loss of access to such methods or therapeutics agents may hinder development of our current or future product candidates, and our business and prospects could be materially harmed.

We currently rely, and expect to continue to rely, on third parties to develop and manufacture our clinical product supplies, and we intend to rely on third parties to produce and process our product candidates, if approved. This reliance increases the risk that we may not have sufficient quantities of our product candidates within our desired timeframes or may not be able to produce such quantities at an acceptable cost or quality level, which could delay, prevent or impair our progress in conducting clinical trials and/or our commercialization efforts.

We do not currently own any facility that may be used as our clinical-scale manufacturing and processing facility and, as a result, we rely, and expect to continue to rely, on third parties for the manufacture of our clinical product supplies and product candidates for our clinical development efforts, as well as for the potential commercial manufacture of our product candidates, if approved, and the related label and packaging activities involved in commercialization. We rely on these third parties to produce our clinical product supplies and product candidates at sufficient quality and quantity to support our development and commercialization efforts. Our reliance on third parties increases the risk that we will have insufficient quantities of our product candidates or that our product candidates will not be produced at an acceptable cost or quality level. If any of the foregoing risks materialize, this could delay, prevent or impair our development or commercialization efforts.

Additionally, even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- regulatory or judicial termination or modification of our agreement with the third party due to the third party's insolvency or winddown, a change in regulations or other reason;
- 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.

If any CDMO with whom we contract fails to or otherwise becomes unable to perform its obligations, we may be forced to manufacture the materials ourselves, for which we may not have the capabilities, technical knowledge or resources, or enter into an agreement with a different CDMO, which we may not be able to do on reasonable terms, if at all. In addition, if we have a material

dispute with any of our CDMOs or should any of our agreements with our CDMOs terminate for any reason, including our agreements with Bachem Americas, Inc. and Heidelberg Pharma, it could disrupt our supply and replacing these CDMOs would likely be difficult. In these scenarios, our clinical trials could be delayed significantly as we establish alternative supply sources.

In some cases, the technical skills required to manufacture our products or product candidates may be unique or proprietary to the original CDMO and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternative supplier, or we may be unable to transfer such skills at all. For example, we are currently in the process of transferring the late-stage manufacturing of amanitin from Heidelberg Pharma to other CDMOs. Whenever changing a CDMO, we will be required to verify that the new CDMO maintains facilities and procedures that comply with quality standards and with all applicable regulations. We will also need to verify, such as through a manufacturing comparability study, that any new manufacturing process will produce our product candidate according to the specifications previously submitted to the FDA or another regulatory authority. We cannot provide any assurance that the technology transfer to another CDMO will be successful in producing our product candidate in sufficient quantities or of acceptable quality, if at all, or that we or another CDMO will produce a comparable product to the satisfaction of the FDA or other comparable regulatory authorities, which could delay, prevent or impair the development or commercialization of our product candidates. In addition, there is typically a transition period when a new CDMO commences work. The delays associated with the verification of a new CDMO could negatively affect our ability to develop product candidates or commercialize our products in a timely manner or within budget. Furthermore, a CDMO may possess technology related to the manufacture of our product candidate that such CDMO owns independently. This would increase our reliance on such CDMO or require us to obtain a license from such CDMO in order to have another CDMO manufacture our product candidates. In addition, changes in manufacturers often involve changes in manufacturing procedures and processes, which could require that we conduct bridging studies between our prior clinical supply used in our clinical trials and that of any new manufacture. We may be unsuccessful in demonstrating the comparability of clinical supplies which could require the conduct of additional clinical trials.

The facilities used by our CDMOs to manufacture our product candidates must be approved and may be inspected by the FDA and other comparable regulatory authorities in connection with the submission of our marketing applications to, and review by, the FDA or other comparable regulatory authorities, or based on the work by these CDMOs for other clinical trial sponsors. While we have contractual relationships with our CDMOs, our oversight of manufacturing activities is limited and we do not and will not control the manufacturing process of, and will be completely dependent on, our CDMOs for compliance with cGMPs and other regulatory requirements in connection with the manufacture of our product candidates. In addition, certain of our CDMOs may themselves rely on other third parties to produce all or a part of our product candidates. If our CDMOs (or any of the third parties upon which they rely) cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or other applicable regulatory authorities in other jurisdictions, they will not be able to secure or maintain regulatory approval for their manufacturing facilities. While we may review the compliance history and performance of our CDMOs, we have no control over the ability of our CDMOs to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or comparable regulatory authorities in other jurisdictions does not approve these facilities for the manufacture of our product candidates, or if the FDA or comparable regulatory authorities withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved. Further, 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, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates, if approved, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect our business and supplies of our product candidates.

Our product candidates may compete with other product candidates and approved 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. Furthermore, given the limited number of available manufacturing slots and the long lead times needed to reserve them, manufacturers require monetary commitments in connection with such reservations as well as fees for changes or cancellations in the reserved manufacturing slots. As a result, we may wait to reserve manufacturing slots until we can be informed by data from the clinical trials of our product candidates, which may be several months from the time we request manufacturing slots. Additionally, limitations on our capital resources and ability to raise additional capital may cause us to delay reserving manufacturing slots. Any significant delay in the supply of clinical materials for our product candidates could considerably delay conducting our clinical trials and potential regulatory approval of our product candidates. Alternatively, we may project when we may need additional clinical material for our product candidates and reserve manufacturing time-slots “at-risk” prior to our product candidates having generated data from their then current clinical trials. Such projections involve risks and uncertainties and may result in additional costs or delays in manufacturing clinical materials for our product candidates when and if we actually need them.

Any contamination in our or our third parties' manufacturing process, shortages of raw materials or reagents or failure of any of our key suppliers to deliver necessary components of our product candidates could result in delays in our clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could materially adversely affect our or our third-party vendors' ability to produce our product candidates on schedule and could therefore harm our results of operations and cause reputational damage.

The raw materials required in our and our third-party vendors' manufacturing processes are derived from biological sources. We cannot assure you that we or our third-party vendors have, or will be able to obtain on commercially reasonable terms, or at all, sufficient rights to these materials derived from biological sources. Such raw materials are difficult to procure and may also be subject to contamination or recall or be of insufficient quality. A material shortage, contamination, recall, or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the clinical and commercial manufacturing of our product candidates, which could materially and adversely affect our operating results and development timelines. We rely on third-party suppliers for the supply and manufacture of certain components of our technology and product candidates, including a single supplier in some cases. Should our ability to procure the necessary components for our product candidates from our suppliers be compromised, our ability to continuously operate would be impaired until an alternative supplier is sourced, qualified and tested, which could delay or limit our ability to produce a clinical and commercial supply of our product candidates and harm our business.

Moreover, if there is a disruption to our manufacturing operations or one or more of our third-party manufacturers' or suppliers' relevant operations, such as due to the impact of the COVID-19 pandemic, including due to staffing shortages or reprioritizations, production slowdowns or stoppages or interruptions in global shipping, the supply of the related product candidate will be delayed until we or such manufacturer or supplier restores the affected facilities or we or they procure alternative manufacturing facilities or sources of supply. Our ability to progress our preclinical and clinical programs could be materially and adversely impacted if any of the third-party suppliers upon which we rely for preclinical and clinical stage product candidate supply were to experience a significant business challenge, disruption or failure due to issues such as financial difficulties or bankruptcy, issues relating to other customers such as regulatory or quality compliance issues, or other financial, legal, regulatory or reputational issues. Additionally, any damage to or destruction of our third-party manufacturers' or suppliers' facilities or equipment may significantly impair our ability to manufacture our product candidates on a timely basis.

If we handle biological materials in a manner that causes injury, we may be liable for damages.

Our research and development activities involve the use of biological and hazardous materials. Although we believe our safety procedures for handling and disposing of these materials complies with federal, state and local laws and regulations, we cannot entirely eliminate the risk of accidental injury or contamination from the use, storage, handling or disposal of these materials. We do not carry specific biological waste insurance coverage or property and casualty and general liability insurance policies that include coverage for damages and fines arising from biological exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended or terminated.

Third-party manufacturers and any third-party collaborators may be unable to successfully scale-up manufacturing of our current or future product candidates in sufficient quality and quantity, which would delay or prevent us from developing our product candidates and commercializing approved products, if any.

In order to conduct clinical trials of MGTA-117, MGTA-145 and our other current and future product candidates, we will need to work with third-party manufacturers to manufacture them in sufficient quantities. We have not yet had our product candidates manufactured or processed to support later stage clinical trials or commercialization, and we may not be able to do so. Our manufacturing partners or our third-party collaborators may be unable to successfully create and support the manufacturing capacity of MGTA-117, MGTA-145 and our other current or future product candidates at the correct scale and within a manufacturing suite intended for pivotal material production, or in a timely or cost-effective manner, or at all. In particular, the amanitin payload of MGTA-117 requires a complex manufacturing process that will need to be further developed for late-stage clinical trials or commercialization. We will make changes as we work to optimize the manufacturing process, and we cannot be sure that even minor changes in the process will result in therapies that are safe and effective. The process of making these changes could delay, prevent or impair the clinical development or commercialization of our product candidates. In addition, quality issues may arise during scale-up activities. If our manufacturing partners or collaborators are unable to successfully scale up the manufacture of our current or future product candidates in sufficient quality and quantity, the development, testing, and clinical trials of that product candidate may be delayed or infeasible, and marketing approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business.

Risks Related to Commercialization, Government Regulation and Competition

We may never obtain FDA approval for any of our product candidates in the U.S., and even if we do, we may never obtain approval for or commercialize any of our product candidates in any other jurisdiction, which would limit our ability to realize their full market potential.

In order to eventually market any of our product candidates in any particular foreign jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a jurisdiction-by-jurisdiction basis regarding safety and efficacy. Approval by the FDA in the U.S., if obtained, does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional preclinical studies or clinical trials which could be costly and time-consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. The foreign regulatory approval process involves all of the risks associated with FDA approval. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of our products will be unrealized.

Failure to obtain marketing approval in foreign jurisdictions would prevent any product candidates we may develop from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.

In order to market and sell any product candidates we may develop in the European Union and many other foreign jurisdictions, we or our third-party collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the U.S. generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the U.S., it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the U.S. on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the U.S. does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our medicines in any jurisdiction, which would materially impair our ability to generate revenue.

Even if we obtain marketing approvals for any product candidates we develop, the terms of approvals and ongoing regulation of our products could require the substantial expenditure of resources and may limit how we manufacture and market our products, which could materially impair our ability to generate revenue.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising, and promotional activities for such medicine, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to quality control, applicable product tracking and tracing requirements, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the medicine may be marketed or to the conditions of approval or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine.

Accordingly, assuming we, or any collaborators we may have, receive marketing approval for one or more product candidates we develop, we, and such collaborators, and our and their contract manufacturers will continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance, and quality control. If we and such collaborators are not able to comply with post-approval regulatory requirements, we and such collaborators could have the marketing approvals for our products withdrawn by regulatory authorities and our, or such collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our business, operating results, financial condition and prospects.

Even if our product candidates are approved by government regulators, the commercial success of any of our product candidates will depend upon the degree of market acceptance by physicians, patients, third-party payors and others in the medical community.

Even with the requisite approvals from the FDA in the U.S., the EMA in the European Union and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of our product candidates as medically necessary, cost-effective and safe. Even before receiving any potential regulatory approval for a product candidate, we may determine that the clinical trial results for a product candidate suggest that it does not have a product profile that would be competitive compared to other therapeutic options. Any product that we develop or commercialize may not have or gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. Efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources, including management time and financial resources, and may not be successful. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy, durability and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- our ability to offer the product for sale at competitive prices;
- the clinical indications for which the product candidate is approved by the FDA or the EMA;
- the product's convenience and ease of administration compared to alternative treatments;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments;
- changes in the standard of care for the targeted indications for the product; and
- sufficient third-party payor coverage and adequate reimbursement.

In addition, we analyze these factors with respect to our product candidates before they are approved by conducting market research. Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched. Further, we may determine not to commercialize a product candidate based on that analysis or based on unfavorable pricing and reimbursement terms. Any current or potential product candidate of ours that does not have a competitive product profile compared to other therapeutic options, including those that obtain regulatory approval but fail to achieve market acceptance or commercial success, would adversely affect our business prospects.

We currently have no marketing and sales organization and have no experience in marketing products. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be able to generate product revenue.

We currently have no sales, marketing or distribution capabilities and have no experience in marketing products. We intend to develop an in-house marketing organization and sales force, which will require significant capital expenditures, management resources and time. We will have to compete with other pharmaceutical and biotechnology companies to recruit, hire, train and retain marketing and sales personnel. If we are unable or decide not to establish internal sales, marketing and distribution capabilities, we will pursue collaborative arrangements regarding the sales and marketing of our products, however, there can be no assurance that we will be able to establish or maintain such collaborative arrangements, or if we are able to do so, that they will have effective sales forces. Any revenue we receive will depend upon the efforts of such third parties, which may not be successful. We may have little or no control over the marketing and sales efforts of such third parties and our revenue from product sales may be lower than if we had commercialized our product candidates ourselves. We also face competition in our search for third parties to assist us with the sales

and marketing efforts of our product candidates. There can be no assurance that we will be able to develop in-house sales and distribution capabilities or establish or maintain relationships with third-party collaborators to commercialize any product in the U.S. or overseas.

Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, if approved, which could make it difficult for us to sell any product candidates or therapies profitably.

The success of our product candidates, if approved, depends on the availability of adequate coverage and reimbursement from third-party payors. For additional information regarding laws and regulations related to reimbursement, see "Item 1. Business – Reimbursement" in our Annual Report on Form 10-K. Because our product candidates represent new approaches to blood and immune reset, we cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from our product candidates or assure that coverage and reimbursement will be available for any product that we may develop. In addition, we plan to develop certain of our product candidates to be used in conjunction with gene therapy treatments that have encountered challenges in obtaining coverage and reimbursement, and such challenges may also affect the coverage and reimbursement we may obtain for our product candidates, or may indirectly impact the commercial potential for our product candidates if the gene therapy treatment which with our product candidate would be used is not adequately covered or reimbursed.

In the U.S. and markets in other countries, patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors are critical to new product acceptance. If government and other health care payers were not to provide adequate coverage and reimbursement levels for any of our products if approved, market acceptance and commercial success could be reduced.

Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drugs and treatments they will cover and the amount of reimbursement. Coverage may be more limited than the purposes for which the medicine is approved by the FDA or comparable foreign regulatory authorities. In the U.S., the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the Department of Health and Human Services, or HHS. CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

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

Further, in the U.S., no uniform policy of coverage and reimbursement for products exists among third-party payors. As a result, obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost-effectiveness data for the use of our products on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. Even if we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate for us to maintain pricing sufficient to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Additionally, third-party payors may not cover, or provide adequate reimbursement for, long-term follow-up evaluations required following the use of product candidates. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates. Because our product candidates may have a higher cost of goods than conventional therapies, and may require long-term follow up evaluations, the risk that coverage and reimbursement rates may be inadequate for us to achieve profitability may be greater. There is significant uncertainty related to insurance coverage and reimbursement of newly approved products. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Moreover, increasing efforts by governmental and third-party payors in the U.S. and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. There has been increasing legislative and enforcement interest in the U.S. with respect to specialty drug pricing practices. Specifically, there have been several recent Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform

government program reimbursement methodologies for drugs. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, cost containment initiatives and additional legislative changes.

Even if we are able to commercialize any product candidates, such products may become subject to unfavorable pricing regulations, third-party reimbursement practices, or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing, and reimbursement for new medicines vary widely from country to country. For additional information regarding these variations from country to country, see "Item 1. Business – Governmental Regulation" and "Item 1. Business – Reimbursement" in our Annual Report on Form 10-K. In the U.S., recently enacted legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a medicine before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a medicine in a particular country, but then be subject to price regulations that delay our commercial launch of the medicine, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the medicine in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if any product candidates we may develop obtain marketing approval.

Our ability to commercialize any medicines successfully also will depend in part on the extent to which reimbursement for these medicines and related treatments will be available from government health administration authorities, private health insurers, and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications.

Individual states in the U.S. have also increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Also, increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products.

We cannot be sure that reimbursement will be available for any medicine that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved medicines, and coverage may be more limited than the purposes for which the medicine is approved by the FDA or similar regulatory authorities outside the U.S. Moreover, eligibility for reimbursement does not imply that any medicine will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. Interim reimbursement levels for new medicines, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the medicine and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost medicines and may be incorporated into existing payments for other services. Net prices for medicines may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of medicines from countries where they may be sold at lower prices than in the U.S.

European Union drug marketing and reimbursement regulations may materially affect our ability to market and receive coverage for our products in the European Member States.

We intend to seek approval to market our product candidates in both the U.S. and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for our product candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the European Union, the pricing of biologics is subject to governmental control and other market regulations which could put pressure on the pricing and usage of our product candidates. In these countries, pricing negotiations with governmental authorities can take considerable time after obtaining marketing approval of a product candidate. In addition, market acceptance and sales of our product candidates will depend significantly on the availability of adequate

coverage and reimbursement from third-party payors for our product candidates and may be affected by existing and future health care reform measures.

Failure to comply with the requirements under European Union and U.K. laws and regulations could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment. For additional information regarding applicable government regulations, see "Item 1. Business – Governmental Regulation" in our Annual Report on Form 10-K.

Historically, products launched in the European Union do not follow price structures of the U.S. and generally prices tend to be significantly lower. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If pricing is set at unsatisfactory levels or if reimbursement of our products is unavailable or limited in scope or amount, our revenues from sales by us or our strategic partners and the potential profitability of any of our product candidates in those countries would be negatively affected.

Foreign governments often impose strict price controls on approved products, which may adversely affect our future profitability in those countries, and recent federal legislation and actions by federal, state and local governments may permit reimportation of drugs from foreign countries into the U.S., including foreign countries where the drugs are sold at lower prices than in the U.S., which could adversely affect our future profitability.

Frequently foreign governments impose strict price controls on newly approved therapeutic products. If we obtain regulatory approval to sell products in foreign countries, we may be unable to obtain a price that provides an adequate financial return on our investment. Furthermore, we may face competition in the U.S. for our development candidates and investigational medicines, if approved, from therapies sourced from foreign countries that have placed price controls on pharmaceutical products. Proponents of drug reimportation may attempt to pass legislation that would directly allow reimportation under certain circumstances. Legislation or regulations allowing the reimportation of drugs, if enacted, could decrease the price we receive for any products that we may develop and adversely affect our future revenues and prospects for profitability.

Ongoing healthcare legislative and regulatory reform measures may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates, and may affect the prices we may set.

In the U.S. and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any products for which we obtain marketing approval. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (1) changes to our manufacturing arrangements; (2) additions or modifications to product labeling; (3) the recall or discontinuation of our products; or (4) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business. For additional information regarding these regulations, statutes or their interpretations, see "Item 1. Business – Governmental Regulation" in our Annual Report on Form 10-K.

Additional laws, and future state and federal healthcare reform measures may be adopted in the future, any of which may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Data collection is governed by restrictive regulations governing the use, processing, and transfer of personal information, and compliance with these regulations could result in additional costs and limitations on our ability to collect and process data. Failure to comply with these regulations could subject us to significant penalties, which may adversely affect our business.

In the event we decide to conduct clinical trials or continue to enroll subjects in our ongoing or future clinical trials in the European Union or the U.K., we may be subject to additional privacy restrictions. The collection, use, storage, transfer, and other processing of personal data, including personal health data, regarding individuals in the European Economic Area is governed, as of May 2018, by the General Data Protection Regulation, or GDPR. Following the U.K.'s withdrawal from the European Union, the data protection obligations of the GDPR continue to apply to U.K.-related processing of personal data in substantially unvaried form under the U.K. General Data Protection Regulation, or U.K. GDPR. However, going forward, there is an increasing risk of divergence in application, interpretation and enforcement of the data protection laws as between the U.K. and the European Union. Achieving and maintaining compliance with the GDPR and the U.K. GDPR is a rigorous and time-intensive process that may increase our cost of doing business or require us to change our business practices, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation, and reputational harm in connection with any future European or U.K. activities. For additional information regarding GDPR and U.K. GDPR, see "Item 1. Business – Governmental Regulation" in our Annual Report on Form 10-K.

In the U.S., the data protection landscape is rapidly growing and evolving, and achieving and maintaining compliance with current and future U.S. state and federal privacy laws will be similarly onerous and may adversely affect our business. For example, if we fail to comply with the California Consumer Protection Act, or CCPA, we could be subject to civil penalties. Further, if we experience a data breach that results in the loss of personal information of California residents, we may be subject to a private right of action under the CCPA. While there are currently exemptions under the CCPA for protected health information that is subject to Health Insurance Portability and Accountability Act, or HIPAA, and for patient information subject to clinical trial regulations, the CCPA may still negatively impact our business activities. There continues to be uncertainty surrounding the enforcement and implementation of the CCPA, which exemplifies the vulnerability of our business to the evolving regulatory environment related to personal data and protected health information.

Additionally, as recently passed U.S. state laws, such as the California Privacy Rights Act come into effect, we may become subject to or affected by new or additional data protection requirements and face increased scrutiny or attention from regulatory authorities. The effects of these laws are potentially significant and may require us to modify our data collection or processing practices and policies and to incur substantial costs and expenses in an effort to comply and increase our potential exposure to regulatory enforcement and/or litigation.

We also anticipate that more states may enact legislation similar to the CCPA, which has prompted a number of proposals for new federal and state-level privacy legislation. Such proposed legislation, if enacted, may add additional complexity, variation in requirements, restrictions and potential legal risk, require additional investment of resources in compliance programs, impact strategies and the availability of previously useful data and could result in increased compliance costs and/or changes in business practices and policies.

Additionally, HIPAA, as amended by the Health Information Technology and Clinical Health Act, or HITECH, and its implementing regulations, imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH makes HIPAA's privacy and security standards directly applicable to "business associates," those independent contractors or agents of covered entities that create, receive, maintain, transmit or obtain protected health information in connection with providing a service on behalf of a covered entity. HITECH also increased the civil and criminal penalties that may be imposed against covered entities, business associates and possibly other persons, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, there may be additional federal, state and non-U.S. laws which govern the privacy and security of health and other personal information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

In addition, there may be additional federal, state and non-U.S. laws which govern the privacy and security of health and other personal information in certain circumstances. These laws may differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. 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 laws and regulations governing the processing of data by healthcare entities. 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, damages, fines, imprisonment, exclusion of drugs from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Ensuring business arrangements comply with applicable laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company's attention away from the business.

Laws and regulations governing international operations may preclude us from developing, manufacturing and selling certain products outside of the U.S. and require us to develop, implement and maintain costly compliance programs.

If we further expand our operations outside of the U.S., we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the U.S. to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all

transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the U.S., or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the U.S., it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the U.S., which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

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

We 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, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. 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. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions. We do not currently carry biological or hazardous waste insurance coverage.

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 or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities.

We are competing against numerous large, established companies that have substantially greater financial, technical, research, manufacturing, marketing, distribution and other resources than us, and our operating results will suffer if we fail to compete effectively.

The pharmaceutical and biopharmaceutical industry is characterized by intense competition and rapid and significant technological changes and advancements. Our potential competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies and universities and other research institutions. Many companies, research institutions and universities are doing research and development work in a number of areas similar to those that we focus on that could lead to the development of new products which could compete with and be superior to our product candidates. We expect technological developments in the pharmaceutical and biopharmaceutical and related fields to occur at a rapid rate, and we believe competition will intensify as advances in these fields are made. Accordingly, we will be required to continue to devote substantial resources and efforts to research and development activities in order to potentially achieve and maintain a competitive position in this field. Products that we develop may become obsolete before we are able to market them or to recover all or any portion of our research and development expenses.

Most of the companies with which we compete have substantially greater financial, technical, research, manufacturing, marketing, distribution and other resources than we do, including staff, experienced marketing and manufacturing organizations, and well-established sales forces. In addition, smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. We will be competing with respect to our products with companies that have significantly more experience and expertise in undertaking preclinical testing and human clinical trials with new or improved therapeutic products and obtaining regulatory approvals of such products. A number of these companies already market and may be in advanced phases of clinical testing of various drugs that will or may compete with our current product candidates or other future potential product candidates. Our competitors may develop or commercialize products more rapidly than we do or with significant advantages over any products we develop. Our competitors may therefore be more successful in commercializing their products than we are, which could adversely affect our competitive position and business.

In addition to larger pharmaceutical or biopharmaceutical companies that may develop different competing technologies or technologies, we will be competing with a number of smaller biotechnology companies. We are aware that collaborations between smaller companies and larger established companies may compete with our programs. Colleges, universities, governmental agencies and other public and private research organizations are becoming more active in seeking patent protection and licensing arrangements to collect royalties for use of technologies that they have developed, some of which may be directly competitive with our programs and product candidates. For additional information regarding our competition, see “Item 1. Business – Competition” in our Annual Report on Form 10-K. In addition, certain gene therapy companies are also developing their own conditioning programs to be used in connection with their therapies.

Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors, either alone or with collaborative partners, may succeed in developing, acquiring or licensing on an exclusive basis drug or biologic products that are more effective, safer, more easily commercialized or less costly than our product candidates. Such competitors may also develop proprietary technologies or secure patent protection that we may need for the development of our technologies and products. We believe the key competitive factors that will affect the development and commercial success of our product candidates are efficacy, safety, tolerability, reliability, convenience of use, price and reimbursement.

Even if we obtain regulatory approval of our product candidates, the availability and price of our competitors’ products could limit the demand and the price we are able to charge for our product candidates. We may not be able to implement our business plan if the acceptance of our product candidates is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our product candidates, or if physicians switch to other new drug or biologic products or choose to reserve our product candidates for use in limited circumstances.

Our competitors include companies focused on developing technologies to improve the distinct steps of stem cell transplant. For additional information regarding our competition, see “Item 1. Business – Competition” in our Annual Report on Form 10-K.

Our product candidates for which we intend to seek approval may face competition from generic drugs or biosimilars sooner than anticipated.

With the enactment of the Biologics Price Competition and Innovation Act of 2009, or BPCIA, an abbreviated pathway for the approval of biosimilar and interchangeable biological products was created. The abbreviated regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as interchangeable based on its similarity to an existing reference product. Under the BPCIA, an application for a biosimilar product cannot be approved by the FDA until 12 years after the original branded product is approved under a BLA. On March 6, 2015, the FDA approved the first biosimilar product under the BPCIA. However, the law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects for our biological products.

We believe that if any of our product candidates are approved as a biological product under a BLA it should qualify for the 12-year period of exclusivity. However, there is a risk that the FDA will not consider any of our product candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Additionally, this period of regulatory exclusivity does not apply to companies pursuing regulatory approval via their own traditional BLA, rather than via the abbreviated pathway. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. Finally, there has been public discussion of

potentially decreasing the period of exclusivity from the current 12 years. If such a change were to be enacted, our product candidates, if approved, could have a shorter period of exclusivity than anticipated.

Risks Related to Intellectual Property

We are highly dependent on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.

In November 2016, we entered into a license agreement with Harvard University, or Harvard, pursuant to which we were granted a worldwide license to research, develop and commercialize one or more therapeutic products under certain conditioning- and mobilization-related patents and patent applications owned or controlled by Harvard. We are dependent on the patents, know-how and proprietary technology licensed from Harvard. In addition, in March 2018, we entered into an exclusive research, development option and license agreement with Heidelberg Pharma Research GmbH, or Heidelberg Pharma, pursuant to which we intend to combine our proprietary antibodies and Heidelberg Pharma's amanitin conjugates platform, including our MGTA-117 product candidate. If we commercialize any products utilizing Heidelberg Pharma's amanitin conjugates platform, we will be dependent on the intellectual property rights we license from Heidelberg Pharma. On August 1, 2022 we entered into an amendment to the exclusive research, development option and license agreement with Heidelberg Pharma mutually clarifying certain performance obligations. Any material disputes with these licensors or termination of these licenses, or a finding that such intellectual property lacks legal effect, could result in the loss of significant intellectual property rights and could harm our ability to commercialize our current or future product candidates.

Certain of our license agreements, including our agreements with Harvard and Heidelberg Pharma, require us to use diligent efforts or meet development thresholds, to maintain the license, including establishing a set timeline for developing and commercializing products. If we fail to comply with any of the obligations under our license agreements, including payment terms and diligence terms, our licensors may have the right to terminate our agreements, in which event we may lose intellectual property rights and may not be able to develop, manufacture, market or sell the products covered by our agreements or may face other penalties under our agreements. In addition, such a termination could result in the licensor reacquiring the intellectual property rights and subsequently enabling a competitor to access the technology. Any such occurrence could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of our license agreements or reduction or elimination of our rights under them may result in our having to negotiate a new or reinstated agreement, which may not be available to us on equally favorable terms, or at all, which may mean we are unable to develop or commercialize the affected product candidate or cause us to lose our rights under the agreement.

Further, 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. Accordingly, material disputes may arise between us and our licensor, or our licensor and its licensors, regarding intellectual property subject to a license agreement, including those relating to:

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

The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, increase what we believe to be our financial or other obligations under the

relevant agreement, or decrease the financial or other benefits we might otherwise receive under the relevant agreement. On August 1, 2022 we entered into an amendment to the exclusive research, development option and license agreement with Heidelberg Pharma mutually clarifying certain performance obligations. If material disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, or are insufficient to provide us the necessary rights to use the intellectual property, we may be unable to successfully develop and commercialize the affected product candidates. If we or any such licensors fail to adequately protect this intellectual property, our ability to commercialize our products could suffer. Any material disputes with our licensors or any termination of the licenses on which we depend could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our commercial success depends on our ability to obtain, maintain and protect our intellectual property and proprietary technology.

Our commercial success depends in large part on our ability to obtain, maintain and protect intellectual property protection through patents, trademarks, and trade secrets in the U.S. and other countries with respect to our proprietary product candidates. If we do not adequately protect our intellectual property rights, competitors may be able to erode, negate or preempt any competitive advantage we may have, which could harm our business and ability to achieve profitability.

To protect our proprietary position, we own and have in-licensed certain issued patents and have filed and may file provisional and non-provisional patent applications in the U.S. or abroad related to our product candidates that are important to our business. Provisional patent applications are not eligible to become issued patents until, among other things, we file a non-provisional patent application within 12 months of the filing of one or more of our related provisional patent applications. If we do not timely file non-provisional patent applications, we may lose our priority date with respect to our provisional patent applications and any patent protection on the inventions disclosed in our provisional patent applications. While we intend to timely file non-provisional patent applications relating to our provisional patent applications, we cannot predict whether any such patent applications will result in the issuance of patents that provide us with any competitive advantage. Moreover, the patent application and approval process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

In some instances, agreements through which we license patent rights may not give us control over patent prosecution or maintenance, so that we may not be able to control which claims or arguments are presented, how claims are amended, and may not be able to secure, maintain, or successfully enforce necessary or desirable patent protection from those patent rights. We have not had and do not have primary control over patent prosecution and maintenance for certain of the patents and patent applications we license, and therefore cannot guarantee that these patents and applications will be prosecuted or maintained in a manner consistent with the best interests of our business. We cannot be certain that patent prosecution and maintenance activities by our licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents. Moreover, some of our in-licensed patents and patent applications are, and our future owned and licensed patents may be, co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us.

If the scope of the patent protection we or our licensors obtain is not sufficiently broad, we may not be able to prevent others from developing and commercializing technology and products similar or identical to ours. The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our licensed patents have, or that any of our pending owned or licensed patent applications that mature into issued patents will include, claims with a scope sufficient to protect our product candidates or otherwise provide any competitive advantage, nor can we assure you that our licenses will remain in force. Other parties have developed or may develop technologies that may be related or competitive with our approach, and may have filed or may file patent applications and may have been issued or may be issued patents with claims that overlap or conflict with our patent applications, either by claiming the same compounds, formulations or methods or by claiming subject matter that could dominate our patent position. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the U.S. Furthermore, patents have a limited lifespan. In the U.S., the natural expiration of a patent is generally twenty years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar to our product candidates. In addition, the patent portfolio licensed to us is, or may be, licensed to third parties, such as outside our field, and such third parties may have certain enforcement rights. Thus, our owned and licensed patents and any patents we own or license in the

future could be put at risk of being invalidated or interpreted narrowly in litigation filed by or against another licensee or in administrative proceedings brought by or against another licensee in response to such litigation or for other reasons.

The patent protection we obtain for our product candidates may not be sufficient to provide us with any competitive advantage or our patents may be challenged.

Our owned and licensed patents and pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our patents by developing similar or alternative technologies or therapeutics in a non-infringing manner. For example, a third party may develop a competitive therapy that provides benefits similar to one or more of our product candidates but falls outside the scope of our patent protection or license rights. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidates could be negatively affected, which would harm our business. Currently, a significant portion of our patents and patent applications are in-licensed, though similar risks would apply to any patents or patent applications that we now own or may own or in-license in the future.

We, or any future partners, collaborators, or licensees, may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, we may miss potential opportunities to strengthen our patent position.

It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If we or our partners, collaborators, licensees, or licensors, whether current or future, fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our partners, collaborators, licensees, or licensors, are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

The patent position of biotechnology and pharmaceutical companies carries uncertainty. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which are dependent upon the current legal and intellectual property context, extant legal precedent and interpretations of the law by individuals. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are characterized by uncertainty.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the U.S., the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Similarly, we cannot be certain that parties from whom we do or may license or purchase patent rights were the first to make relevant claimed inventions, or were the first to file for patent protection for them. If third parties have filed prior patent applications on inventions claimed in our patents or applications that were filed on or before March 15, 2013, an interference proceeding in the U.S. can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such prior applications after March 15, 2013, a derivation proceeding in the U.S. can be initiated by such third parties to determine whether our invention was derived from theirs.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our owned and licensed patents or pending patent applications may be challenged in the courts or patent offices in the U.S. and abroad. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found. If such prior art exists, it may be used to invalidate a patent, or may prevent a patent from issuing from a pending patent application. For example, such patent filings may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or to other patent offices around the world. Alternately or additionally, we may become involved in post-grant review procedures, oppositions, derivation proceedings, *ex parte* reexaminations, *inter partes* review, supplemental examinations, or interference proceedings or challenges in district court, in the U.S. or in various foreign patent offices, including both national and regional, challenging patents or patent applications in which we have rights, including patents on which we rely to protect our business. An adverse determination in any such challenges may result in loss of the patent or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, or in denial of the patent application or loss or reduction in the scope of one or more claims of the

patent application, any of which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

Pending and future patent applications may not result in patents being issued that protect our business, in whole or in part, or which effectively prevent others from commercializing competitive products. Competitors may also be able to design around our patents. 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. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the U.S. For example, patent laws in various jurisdictions, including significant commercial markets such as Europe, restrict the patentability of methods of treatment of the human body more than U.S. law does. Any of these outcomes could have a material adverse effect on our ability to generate revenue.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our future development partners will be successful in protecting our product candidates by obtaining and defending patents. These risks and uncertainties include the following:

- the USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;
- our competitors, many of whom have substantially greater resources and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use, and sell our potential product candidates;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the U.S. for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- countries other than the U.S. may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates.

Issued patents that we have, may obtain or license may not provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting Abbreviated New Drug Applications to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors are competing in a non-infringing manner. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

In addition to the protection afforded by patents, we rely upon trade secret protection, know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our contractors, collaborators, scientific advisors, employees and consultants and invention assignment agreements with our consultants and employees. However, we may not obtain these agreements in all circumstances, and individuals with whom we have these agreements may not comply with their terms. The assignment of intellectual property rights under these agreements may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. In addition, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements despite the existence of confidentiality agreements and other

contractual restrictions. Monitoring unauthorized uses and disclosures is difficult and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the contractors, collaborators, scientific advisors, employees and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets. Enforcing a claim against a third party that illegally obtained and is using our trade secrets, like patent litigation, is expensive and time-consuming and the outcome is unpredictable. In addition, courts outside the U.S. are sometimes less willing or unwilling to protect trade secrets.

Moreover, our trade secrets could otherwise become known or be independently discovered by our competitors or other third parties. Competitors and other third parties could purchase our product candidates and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If our trade secrets are not adequately protected or sufficient to provide an advantage over our competitors, our competitive position could be adversely affected, as could our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets.

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

Our registered or unregistered trademarks or trade names may be challenged, infringed, circumvented, declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. If we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our business, financial condition, results of operations and prospects.

Third-party claims of intellectual property infringement, misappropriation or other violations may prevent or delay our product discovery and development efforts and have a material adverse effect on our business.

Our commercial success depends in part on our avoiding infringement, misappropriation and other violations of the patents and proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. Recently, under U.S. patent reform, new procedures including *inter partes* review and post grant review have been implemented. As stated above, this reform will bring uncertainty to the possibility of challenge to our patents in the future. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others.

Third parties may assert that we are employing their proprietary technology without authorization. For example, we are aware of patents and a patent application owned by a third party with claims that could be construed to cover MGTA-117. The third-party owner of these patents and patent application may seek to allege that our development and commercialization of MGTA-117 infringes their patent rights and file a patent infringement lawsuit against us in the future. While we believe we would have valid defenses against any such allegation or lawsuit, such defenses may be unsuccessful. In this regard, patents issued in the U.S. by law enjoy a presumption of validity that can be rebutted only with evidence that is "clear and convincing," a heightened standard of proof. There may also be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, or any final product itself, the holders of any such patents

may be able to block our ability to commercialize the product candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy or patient selection methods, the holders of any such patent may be able to block our ability to develop and commercialize the product candidate unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all. Even if we obtained such a license, it may only be non-exclusive, which would permit third parties to use the same intellectual property and compete with us. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, we may be unable to commercialize our product candidates or such efforts may be impaired or delayed, which could in turn significantly harm our business.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. We may not have sufficient resources to bring these actions to a successful conclusion. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock.

In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses and access to intellectual property owned or controlled by third parties in order to advance our research or allow commercialization of our product candidates. We may fail to obtain these licenses and/or access to such intellectual property at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our product candidates, which could harm our business significantly. Any of the foregoing may have a material adverse effect on our business, financial condition, results of operations and prospects.

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

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market earlier than would otherwise have been the case, which would have a material adverse effect on our business.

Some intellectual property that we have in-licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements and a preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.

Many of the intellectual property rights we have licensed are generated through the use of U.S. government funding and are therefore subject to certain federal regulations. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980, or Bayh-Dole Act. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (1) adequate steps have not been taken to commercialize the invention; (2) government action is necessary to meet public health or safety needs; or (3) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The U.S. government also has the right to take title to these inventions if we, or the applicable licensor, fail to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. In addition, the U.S. government requires that any

products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the U.S. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the U.S. or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

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

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings. Any of the foregoing may have a material adverse effect on our business, financial condition, results of operations and prospects.

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

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the U.S. has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the case, *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.,* the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. In addition, the case *Amgen Inc. v. Sanofi* affects the way antibody claims are examined and litigated. We cannot predict how future decisions by the courts, the Congress or the USPTO may impact the value of our patents.

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

Filing, prosecuting, maintaining, defending and enforcing patents on our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the U.S. can be less extensive than those in the U.S. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the U.S. Consequently, we may not be able to prevent third parties from practicing our inventions in all

countries outside the U.S., or from selling or importing products made using our inventions in and into the U.S. or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own drugs and may export otherwise infringing drugs to territories where we have patent protection, but enforcement rights are not as strong as those in the U.S. These drugs may compete with our product candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of some countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful.

Many countries have compulsory licensing laws under which a patent owner may be compelled under specified circumstances to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license, which could adversely affect our business, financial condition, results of operations, and prospects.

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

Patents have a limited lifespan. In the U.S., if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest filing date of a non-provisional application to which the patent claims priority. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product candidate, we may be open to competition from competitive medications, including generic medications. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing product candidates similar or identical to ours.

Depending upon the timing, duration and conditions of any FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, and similar legislation in the European Union. The Hatch-Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. However, we may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. Only one patent per approved product can be extended, the extension cannot extend the total patent term beyond 14 years from approval and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for the applicable product candidate will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced. Further, if this occurs, our competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case, and our competitive position, business, financial condition, results of operations and prospects could be materially harmed.

Third parties may assert that our employees or consultants have wrongfully used or disclosed confidential information or misappropriated trade secrets.

We employ individuals who were previously employed at universities or other biopharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of a former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or

personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

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

We may be subject to claims that former employees, collaborators or other third parties have an interest in our owned patent rights, trade secrets or other intellectual property as an inventor or co-inventor. For example, a third party may assert claims against us arising out of conflicting obligations of employees, consultants or others who are involved in developing our product candidates or other technologies. Litigation may be necessary to defend against these and other claims challenging inventorship or our ownership of our owned patent rights, trade secrets or other intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to our product candidates and other technologies. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Intellectual property rights do not necessarily address all potential threats.

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

- others may be able to make products that are similar to any product candidates we may develop or utilize similar technology but that are not covered by the claims of the patents that we license or own;
- we, or our current or future licensors might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or own;
- we, or our current or future licensors might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending owned or licensed patent applications or those that we may own or license in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may harm our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

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

Risks Related to Our Collaborations with Third Parties

We currently depend, and may in the future continue to depend, on collaborations with third parties for the research, development, and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates and our business may be adversely affected.

We currently depend, and may in the future continue to depend, on third-party collaborators for the research, development, and commercialization of certain of the product candidates we may develop. For example, we are working collaboratively with bluebird bio, Inc. for our Phase 2 trial of MGTA-145 plus plerixafor for mobilization and collection of stem cells in patients with sickle cell disease, AVROBIO, Inc., or AVROBIO, to evaluate the potential utility of MGTA-117 for conditioning patients before they receive one of AVROBIO's investigational lentiviral gene therapies, and Beam Therapeutics, or Beam, to evaluate the potential utility of MGTA-117 for conditioning of patients with sickle cell disease and beta-thalassemia receiving Beam's base editing therapies. In our

current collaboration arrangements, and those we may enter into in the future, we have or will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we may seek to develop with them. Our ability to develop MGTA-117 as a conditioning agent for patients before receiving gene therapy, or MGTA-145 plus plerixafor for mobilization and collection of stem cells in patients with sickle cell disease, and generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements, as well as the success of the collaborators' underlying therapies. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our research programs or any product candidates we may develop pose certain risks to us, including the following:

- Collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations.
- Collaborators may not pursue development and commercialization of any product candidates we may develop or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus, available funding or external factors such as an acquisition that diverts resources or creates competing priorities.
- Collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing.
- A collaborator's product candidate may have a safety or efficacy profile that would impact the collaborator's ability to continue to pursue the development and commercialization of its product candidate which in turn would negatively impact our ability to continue to pursue the development and commercialization of our product candidate.
- Collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our medicines or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours.
- Collaborators with marketing and distribution rights to one or more medicines may not commit sufficient resources to the marketing and distribution of such medicine or medicines.
- Collaborators may not properly obtain, maintain, enforce, or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation.
- Material disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of our medicines or product candidates or that result in costly litigation or arbitration that diverts management attention and resources.
- We may lose certain valuable rights under circumstances identified in our collaborations, including if we undergo a change of control.
- Collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.
- Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program under such collaboration could be delayed, diminished, or terminated.
- Current or future collaborators, including in the gene therapy space, may be unable to financially partner with us to develop our product candidates due to the current challenging conditions in the financial markets and their limited ability to raise capital.
- Collaborators may be unable to survive in the current challenging economic environment, and as a result they may be forced to terminate their business operations, including termination of the performance of their collaboration agreements with us.

If our collaborations do not result in the successful development and commercialization of products, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its

agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval, and commercialization described in this Quarterly Report on Form 10-Q apply to the activities of our collaborators.

We have in the past and may in the future decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of any product candidates we may develop. These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of several factors. If we license rights to any product candidates we or our collaborators may develop, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

We may not be successful in finding strategic collaborators for continuing development of certain of our product candidates or successfully commercializing or competing in the market for certain indications.

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 timely capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may 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. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. The terms of any additional collaborations or other arrangements that we may establish may not be favorable to us.

We may also be restricted under existing collaboration agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

In addition, any collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration could adversely affect us financially and could harm our business reputation.

If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our product development and research programs and the potential commercialization of any product candidates we may develop will require substantial additional cash to fund expenses. For some of the product candidates we may develop, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the U.S., the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

If any party to which we have outsourced certain functions fails to perform its obligations under agreements with us, the development and commercialization of our product candidates and any future product candidates could be delayed or terminated.

To the extent that we rely on third party individuals or other companies to manage the day-to-day conduct of our clinical trials or to manufacture, sell or market our current product candidates or any future product candidates, we will be dependent on the timeliness and effectiveness of their efforts. If a clinical research management organization that we might utilize is unable to allocate sufficient qualified personnel to our trials or if the work performed by it does not fully satisfy the rigorous requirements of the FDA, we may encounter substantial delays and increased costs in completing our clinical trials. If a firm producing humanized forms of our molecular antibody product candidates or a manufacturer of the raw material or finished product for our clinical trials is unable to meet our time schedules or cost parameters, the timing of our clinical trials and development of our product candidates may be adversely affected. Any manufacturer that we select may encounter difficulties in scaling-up the manufacture of new products in commercial quantities, including problems involving product yields, product stability or shelf life, quality control, adequacy of control procedures and policies, compliance with FDA regulations and the need for further FDA approval of any new manufacturing processes and facilities. The manufacture of clinical supplies for trials and commercial quantities of our current product candidates and any future product candidates are likely to be inherently more difficult and costly than typical chemical pharmaceuticals. This could delay commercialization of any of our product candidates, if approved, or reduce the profitability of these candidates for us. If any of these occur, the development and commercialization of our product candidates could be delayed, curtailed or terminated because we may not have sufficient financial resources or capabilities to continue such development and commercialization on our own.

Risks Related to Employee Matters, Managing Growth and Other Risks Related to Our Business

The COVID-19 pandemic or any future pandemic, epidemic or outbreak of any other highly infectious disease could have a material adverse effect on our business, financial condition and results of operations.

The COVID-19 pandemic, including the emergence of various variants, has caused, and could continue to cause, widespread disruptions to the U.S. and global economy and has contributed to significant volatility and negative pressure in financial markets. The extent to which the COVID-19 pandemic, or any future pandemic, epidemic or outbreak of any highly infectious disease, impacts our business, financial condition and results of operations will depend on future developments, which are uncertain and cannot be predicted with confidence, including the scope, severity and duration of such pandemic, the emergence and characteristics of new variants, the actions taken to contain the pandemic or mitigate its impact, including the adoption, administration and effectiveness of available COVID-19 vaccines, and the direct and indirect economic effects of the pandemic and containment measures, among others. The rapid development and fluidity of this situation precludes any prediction as to the full adverse impact of the COVID-19 pandemic. Nevertheless, the COVID-19 pandemic has affected, and may continue to adversely affect, our business, financial condition and results of operations, and it has had, and may continue to have, the effect of heightening many of the risks described in this Quarterly Report on Form 10-Q, including but not limited to the following:

- The COVID-19 pandemic has had, and will likely continue to have, an adverse impact on various aspects of our ongoing and planned clinical trials, and preclinical studies.
- Other potential impacts of the COVID-19 pandemic on our various clinical trials include impacts on patient dosing and study monitoring, which may be paused or delayed due to changes in policies at various clinical sites; federal, state, local or foreign laws, rules and regulations, including quarantines or other travel restrictions; the prioritization of healthcare resources toward pandemic efforts, including diminished attention from physicians serving as our clinical trial investigators and reduced availability of site staff supporting the conduct of our clinical trials; and interruption or delays in

the operations of the FDA, among other reasons related to the COVID-19 pandemic. If the COVID-19 pandemic continues, other aspects of our clinical trials will likely be adversely affected, delayed or interrupted, including, for example, site initiation, patient recruitment and enrollment, availability of clinical trial materials and data analysis. Some patients and clinical investigators may not be able to comply with clinical trial protocols and patients may choose to withdraw from our studies or we may choose to, or be required to, pause enrollment and or patient dosing in our ongoing clinical trials in order to preserve health resources and protect trial participants. It is unknown how long these pauses or disruptions could continue.

- We currently rely on third parties, including CROs, CDMOs, and other contractors and consultants to, among other things, conduct our preclinical and clinical trials, manufacture raw materials, manufacture and supply our product candidates, ship investigational drugs and clinical trial samples, perform quality testing and supply other goods and services to run our business. If any such third party is adversely impacted by restrictions resulting from the COVID-19 pandemic, including staffing shortages, production slowdowns and disruptions in delivery systems, our supply chain may be disrupted, which could limit our ability to manufacture our product candidates for our clinical trials and conduct our research and development operations.
- We have established a hybrid work-from-home policy for all employees, as well as safety measures for those using our offices and laboratory facilities that are designed to comply with applicable federal, state and local guidelines instituted in response to the COVID-19 pandemic. Our increased reliance on personnel working from home may negatively impact productivity, or disrupt, delay, or otherwise adversely impact our business. In addition, this could increase our cyber security risk, create data accessibility concerns and make us more susceptible to communication disruptions, any of which could adversely impact our business operations or delay necessary interactions with local and federal regulators, ethics committees, manufacturing sites, research or clinical trial sites and other important agencies and contractors.
- Our employees and contractors conducting non-business critical research and development activities may not be able to access our laboratory for an extended period of time as a result of the COVID-19 pandemic and the possibility that governmental authorities further modify current restrictions. This could delay timely completion of preclinical activities, including completing Investigational New Drug, or IND, enabling studies or our ability to select future development candidates, and initiation of additional clinical trials for our other product candidates.
- Certain government agencies, such as health regulatory agencies and patent offices, within the U.S. or internationally have experienced, and may continue to experience, disruptions in their operations as a result of the COVID-19 pandemic. The FDA and comparable foreign regulatory agencies may have slower response times or be under-resourced to continue to monitor our clinical trials and, as a result, review, inspection and other timelines may be materially delayed. It is unknown how long these disruptions could continue. 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. For example, regulatory authorities may require that we not distribute a product candidate lot until the relevant agency authorizes its release. Such release authorization may be delayed as a result of the COVID-19 pandemic, which would likely result in delays to our ongoing clinical trials.
- The trading prices for our common stock and those of other biopharmaceutical companies have been highly volatile, partly due to the COVID-19 pandemic. As a result, we may face difficulties raising capital through sales of our common stock or such sales may be on unfavorable terms. In addition, a recession, depression or other sustained adverse market event resulting from the COVID-19 pandemic could materially and adversely affect our business and the value of our common stock.

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As of September 30, 2022, we had 66 full-time employees. As our development, manufacturing and commercialization plans and strategies develop, and as we continue to operate as a public company, we expect to need additional managerial, technical, operational, sales, marketing, financial and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our internal development efforts effectively, including the clinical, FDA and international regulatory review process for our product candidates, while complying with our contractual obligations to contractors and other third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to develop, manufacture and commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert financial and

other resources, and a disproportionate amount of their attention away from day-to-day activities in order to devote a substantial amount of time, to managing these growth activities.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services, including core aspects of regulatory approval, clinical management and manufacturing. We cannot assure you that the services of independent organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements. We may also overextend consultants in certain roles. If we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. We cannot assure you that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

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

If we lose key personnel, or if we fail to recruit additional highly skilled personnel, our ability to develop our product candidates will be impaired and our business may be harmed.

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel with particular subject matter expertise. We are highly dependent on our management, particularly our Chief Executive Officer, the members of our executive team as well as key scientific and medical personnel employees. The loss of the services of any of our executive officers, key employees, and scientific and medical advisors, and our inability to find suitable replacements, could result in delays in the development of our product candidates and harm our business. Unless we are able to replace departed employees effectively, we may require current employees to fill certain roles, and this could overextend their responsibilities. As a result, we may experience increased turnover due to employees being overworked. Additionally, employees may be unable to perform these multiple roles effectively due to time and resource constraints.

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

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

Additionally, if we are unable to retain key personnel, we may be required to cover the roles previously performed by such employees with consultants. These consultants may lack the same skills and performance of departed employees and, as a result, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business.

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

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs, and sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational, and financial systems, expand our facilities, and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expected expansion of our operations or recruit and train additional qualified personnel. Moreover, the expected physical expansion of our operations may lead to significant costs and

may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit the development and commercialization of our product candidates.

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk if we commercialize any products. For example, we may be sued if our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical 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. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our products;
- injury to our reputation;
- withdrawal of clinical trial participants and inability to continue clinical trials;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any product candidate; and
- a decline in our share price.

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop, alone or with collaborators. Although we currently carry clinical trial insurance, the amount of such insurance coverage may not be adequate, we may be unable to maintain such insurance, or we may not be able to obtain additional or replacement insurance at a reasonable cost, if at all. Our insurance policies may also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

Our internal computer systems, or those of our collaborators, other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from future clinical trials 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 or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed.

We could be subject to risks caused by misappropriation, misuse, leakage, falsification or intentional or accidental release or loss of information maintained in the information systems and networks of our company and our vendors, including personal information of our employees and study subjects, and company and vendor confidential data. In addition, outside parties may attempt to penetrate our systems or those of our vendors or fraudulently induce our personnel or the personnel of our vendors to disclose sensitive information in order to gain access to our data and/or systems. We may experience threats to our data and systems, including malicious codes and viruses, phishing and other cyber-attacks. The number and complexity of these threats continue to increase over time. If a material breach of our information technology systems or those of our vendors occurs, the market perception of the effectiveness of our security measures could be harmed and our reputation and credibility could be damaged. We could be required to expend significant amounts of money and other resources to repair or replace information systems or networks. In addition, we could be subject to regulatory actions and/or claims made by individuals and groups in private litigation involving privacy issues related to data collection and use practices and other data privacy laws and regulations, including claims for misuse or inappropriate disclosure of data, as well as unfair or deceptive practices. Although we develop and maintain systems and controls designed to prevent these events from occurring, and we have a process to identify and mitigate threats, the development and maintenance of these systems, controls and processes is costly and requires ongoing monitoring and updating as technologies change and efforts to overcome security measures become increasingly sophisticated. Moreover, despite our efforts, the possibility of these events occurring cannot be eliminated entirely. As we outsource more of our information systems to vendors, engage in more electronic transactions with payors and patients, and rely more on cloud-based information systems, the related security risks will increase and we will need to expend additional resources to protect our technology and information systems. In addition, there can be no assurance that our internal information technology systems or those of our third-party contractors, or our consultants' efforts to implement adequate security and control measures, will be sufficient to protect us against breakdowns, service disruption, data deterioration or loss in the event of a system malfunction, or prevent data from being stolen or corrupted in the event of a cyberattack, security breach, industrial espionage attacks or insider threat attacks which could result in financial, legal, business or reputational harm.

We and the third parties with whom we work are increasingly utilizing social media tools as a means of communication both internally and externally, and noncompliance with applicable requirements, policies or contracts due to social media use or negative posts or comments could have an adverse effect on our business.

Social media is increasingly being used to communicate about our clinical development programs and the diseases our therapeutics are being developed to treat, and we intend to utilize appropriate social media in connection with our commercialization efforts following approval of our product candidates, if any. Social media practices in the biopharmaceutical industry continue to evolve and regulations and regulatory guidance relating to such use are evolving and not always clear. In addition, our employees or third parties with whom we contract, such as our CROs or CDMOs, may knowingly or inadvertently make use of social media in ways that may not comply with legal or contractual requirements, which may give rise to liability, lead to the loss of trade secrets or other intellectual property or result in public exposure of personal information of our employees, clinical trial patients and others or information regarding our product candidates or clinical trials along with the potential for litigation related to off-label marketing or other prohibited activities. For example, clinical trial patients may use social media channels to comment on their experience in an ongoing blinded clinical trial or to report an alleged adverse event. When such disclosures occur, there is a risk that trial enrollment may be adversely impacted, we fail to monitor and comply with applicable adverse event reporting obligations or that we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product candidates. There is also a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website. Furthermore, negative posts or comments about us or our product candidates on social media could seriously damage our reputation, brand image and goodwill. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions or incur other harm to our business.

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

As of December 31, 2021, we had net operating loss carryforwards for federal income tax purposes of $247.2 million, of which $17.5 million begin to expire in 2035 and $229.7 million can be carried forward indefinitely. As of December 31, 2021, we had net operating loss carryforwards for state income tax purposes of $247.4 million which begin to expire in 2035. As of December 31, 2021, we also had available research and orphan drug tax credit carryforwards for federal and state income tax purposes of $10.3 million and $2.7 million, respectively, which begin to expire in 2035 and 2030, respectively. These net operating loss carryforwards and research and orphan drug tax credit carryforwards could expire unused and be unavailable to offset future income tax liabilities. In addition, in general, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-change net operating losses or tax credits to offset future taxable income or taxes. For these purposes, an ownership change generally occurs where the aggregate stock ownership of one or more stockholders or groups of stockholders who own at least 5% of a corporation's stock increases its ownership by more than 50 percentage points over its lowest ownership percentage (by value) within a rolling three-year period. Utilization of our net operating loss carryforwards and research and orphan drug tax credit carryforwards may be subject to a substantial annual limitation under

Section 382 and 383 of the Code due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. The Company has not conducted a formal study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If an ownership change has occurred or does occur in the future, the amount of net operating loss and tax credit carryforwards presented in our financial statements could be limited or expire unutilized.

Risks Related to Our Common Stock

An active trading market for our common stock may not be sustained.

In June 2018, we closed our IPO. Prior to our IPO, there was no public market for our common stock. Although we have completed our IPO and shares of our common stock are listed and trading on the Nasdaq Global Market, an active trading market for our shares may not be sustained. If an active market for our common stock does not continue, it may be difficult for our stockholders to sell their shares without depressing the market price for the shares, sell their shares at or above the prices at which they acquired their shares or sell their shares at the time they would like to sell. Any inactive trading market for our common stock may also impair our ability to raise capital to continue to fund our operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

The trading price of our common stock has been, and will likely continue to be, highly volatile.

The trading price of our common stock may be highly volatile. The stock market in general, and the market for smaller pharmaceutical and biotechnology companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the purchase price, and you may lose some or all of your investment. The market price for our common stock may be influenced by many factors, including:

- the success of existing or new competitive products or technologies;
- regulatory actions with respect to our product candidates or our competitors' products and product candidates;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- the timing and results of preclinical studies for any of our product candidates;
- the timing and results of clinical trials of MGTA-117, MGTA-145 and any other product candidates;
- commencement or termination of collaborations for any of our current and future programs and product candidates;
- failure or discontinuation of any of our development programs;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the U.S. and other countries;
- developments or material disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results or development timelines;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or other stockholders;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;

- disruptions to political, governmental or regulatory systems, including shutdowns of the government and its agencies;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

We are an "emerging growth company" and a "smaller reporting company," and the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.

We are an emerging growth company, as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years following the year in which we completed our IPO, although circumstances could cause us to lose that status earlier. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the closing of our IPO, (b) in which we have total annual gross revenue of at least $1.235 billion or (c) in which we are deemed to be a large accelerated filer, as defined in Rule 12b-2 under the Exchange Act, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. We have elected not 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 will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (1) irrevocably elect to "opt out" of such extended transition period or (2) no longer qualify as an emerging growth company.

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future. Accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment.

We have never declared nor paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. In addition, the terms of any future debt or credit agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Our executive officers, directors and principal stockholders, together with their respective affiliates, beneficially owned approximately 59% of our capital stock as of September 30, 2022. This concentration of ownership control could delay, defer or prevent a change in control, entrench our management or the board of directors, or impede a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

Provisions in our corporate charter documents and provisions under Delaware law may prevent or frustrate attempts by our stockholders to change our management or hinder efforts to acquire a controlling interest in us.

Provisions in our corporate charter and our by-laws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is

responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that all members of the board are not elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call a special meeting of stockholders;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 66.67% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or by-laws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns 15% or more of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired 15% or more of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. This could discourage, delay or prevent someone from acquiring us or merging with us, whether or not it is desired by, or beneficial to, our stockholders. This could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in the best interest of our stockholders. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Our by-laws provide that, unless we consent in writing to the selection of an alternative forum, certain designated courts will be the sole and exclusive forum for certain legal actions between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or agents.

Our by-laws provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware is the sole and exclusive forum for state law claims for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of or based on a breach of a fiduciary duty owed by any of our current or former directors, officers, or other employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our charter or our by-laws, or (iv) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein, which we refer to herein as the "Delaware Forum Provision." The Delaware Forum Provision will not apply to any causes of action arising under the Securities Act and the Exchange Act. Our by-laws further provide that, unless we consent in writing to an alternative forum, the United States District Court for the District of Massachusetts will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, which we refer to herein as the "Federal Forum Provision." We have chosen the United States District Court for the District of Massachusetts as the exclusive forum for such causes of action because our principal executive offices are located in Cambridge, Massachusetts. In addition, our by-laws provide that any person or entity purchasing or otherwise acquiring any interest in shares of our capital stock is deemed to have notice of and consented to the foregoing Delaware Forum Provision and Federal Forum Provision; provided, however, that stockholders cannot and will not be deemed to have waived our compliance with the U.S. federal securities laws and the rules and regulations thereunder.

We recognize that the Delaware Forum Provision and the Federal Forum Provision may impose additional litigation costs on stockholders in pursuing any such claims, particularly if the stockholders do not reside in or near the State of Delaware or the Commonwealth of Massachusetts, as applicable. Additionally, the forum selection clauses in our by-laws may limit our stockholders' ability to bring a claim in a judicial forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders.

While the Delaware Supreme Court and other states courts have upheld the validity of federal forum selection provisions purporting to require claims under the Securities Act be brought in federal court, there is uncertainty as to whether other courts will

enforce our Federal Forum Provision. If the Federal Forum Provision is found to be unenforceable, we may incur additional costs associated with resolving such matters and the Federal Forum Provision may also impose additional litigation costs on stockholders who assert the provision is not enforceable or invalid. The Court of Chancery of the State of Delaware or the U.S. District Court for the District of Massachusetts, as applicable, may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments may be more or less favorable to us than our stockholders.

General Risk Factors

Changes in tax law could adversely affect our business and financial condition.

The rules dealing with U.S. federal, state, and local income taxation are constantly under review by persons involved in the legislative process and by the U.S. Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge investors to consult with their legal and tax advisers regarding the implications of changes in tax laws on an investment in our common stock.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. 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 stock.

We are required to disclose changes made in our internal controls and procedures on a quarterly basis and our management will be required to assess the effectiveness of these controls annually. However, for as long as we are an "emerging growth company" under the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act. We could be an "emerging growth company" up until December 31, 2023. An independent assessment of the effectiveness of our internal controls over financial reporting could detect problems that our management's assessment might not. Undetected material weaknesses in our internal controls over financial reporting could lead to financial statement restatements and require us to incur the expense of remediation.

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

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

At September 30, 2022, we had $128.3 million of cash, cash equivalents and marketable securities. While we are not aware of any downgrades, material losses, or other significant deterioration in the fair value of our cash equivalents or marketable securities since September 30, 2022, no assurance can be given that further deterioration of the global credit and financial markets would not

negatively impact our current portfolio of cash equivalents or our ability to meet our financing objectives. Furthermore, our stock price may decline due in part to the volatility of the stock market and the general economic downturn.

Securities class action and derivative lawsuits and other legal proceedings are often brought against companies such as ours that could result in substantial costs and divert management's attention, and our insurance policies may be inadequate and potentially expose us to unrecoverable risks.

Securities class action and derivative lawsuits and other legal proceedings are often brought against companies following a decline in the market price of their securities. This risk is especially relevant for biopharmaceutical companies, which have experienced significant stock price volatility in recent years. As a result, we may be more susceptible to these types of lawsuits and legal proceedings than other companies with more stable security prices. In connection with any litigation or other legal proceedings, we could incur substantial costs, and such costs and any related settlements or judgments may not be covered by insurance. We could also suffer an adverse impact on our reputation and a diversion of management's attention and resources, which could have a material adverse effect on our business.

We have limited director and officer insurance and commercial insurance policies. Any significant insurance claims would have a material adverse effect on our business, financial condition and results of operations. Insurance availability, coverage terms and pricing continue to vary with market conditions and insurance coverage is increasingly expensive. We have observed rapidly changing conditions in the insurance markets relating to nearly all areas of traditional corporate insurance and such conditions have resulted in higher premium costs, higher policy deductibles and lower coverage limits. We endeavor to obtain appropriate insurance coverage for insurable risks that we identify, however, we may fail to correctly anticipate or quantify insurable risks, we may not be able to obtain appropriate insurance coverage and insurers may not respond as we intend to cover insurable events that may occur. If we are unable to obtain insurance at an acceptable cost or otherwise protect against potential class action and derivative lawsuits and other legal proceedings or claims often brought against companies following a decline in the market price of their securities, we will be exposed to significant liabilities that may materially and adversely affect our business and financial position.

Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.

Our operations, and those of our CDMOs, our CROs and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. Our ability to obtain clinical supplies of our product candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption.

Our internal computer systems, or those used by our CDMOs, CROs or other contractors or consultants, may fail or suffer security breaches.

Despite the implementation of security measures, our internal computer systems and those of our future CDMOs, future CROs and other contractors and consultants are vulnerable to damage from computer viruses and unauthorized access. Furthermore, because the techniques used to obtain unauthorized access to, or to sabotage, systems change frequently and often are not recognized until launched against a target, we may be unable to anticipate these techniques or implement adequate preventative measures. We may also experience security breaches that may remain undetected for an extended period. If such a system failure or security breach were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we may rely on third parties for the manufacture of our product candidates and to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, including potential lawsuits from patients, collaborators, employees and/or stockholders, and the further development and commercialization of our product candidates could be delayed.

Our employees, independent contractors, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of employee fraud or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to: comply with the laws of the FDA and other similar foreign regulatory bodies, provide true, complete and accurate information to the FDA and other similar foreign regulatory bodies, comply with manufacturing standards we have established, comply with

healthcare fraud and abuse laws in the U.S. and similar foreign fraudulent misconduct laws, report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of any of our product candidates and begin commercializing those products in the U.S., our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. These laws may impact, among other things, our current activities with principal investigators and research patients, as well as proposed and future sales, marketing and education programs.

Our relationships with healthcare providers and physicians and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the U.S. and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute, the False Claims Act, laws and regulations related to the reporting of payments to physicians and teaching hospitals, and HIPAA, which may constrain the business or financial arrangements and relationships through which such companies sell, market and distribute pharmaceutical products. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed 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, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials. For additional information, see “Item 1. Business – Governmental Regulation” in our Annual Report on Form 10-K. The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies often scrutinize interactions between healthcare companies and healthcare providers, and this scrutiny has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company’s attention from the business.

The failure to comply with any of these laws or regulatory requirements subjects entities to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in civil, criminal and administrative penalties, damages, fines, disgorgement, individual imprisonment, possible exclusion from participation in federal and state funded healthcare programs, contractual damages and the curtailment or restricting of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws. Any action for violation of these laws, even if successfully defended, could cause a pharmaceutical manufacturer to incur significant legal expenses and divert management’s attention from the operation of the business. Prohibitions or restrictions on sales or withdrawal of future marketed products could materially affect business in an adverse way. In connection with our IPO, we adopted a code of business conduct and ethics, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations, guidance or case law interpreting applicable fraud and abuse or other healthcare laws and 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, including the imposition of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of noncompliance with these laws, contractual damages, reputational harm, diminished profits and future earnings and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our product candidates outside the U.S. will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

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

As a public company, and particularly after we are no longer an “emerging growth company,” we will incur significant legal, accounting and other expenses that we did not incur as a private company, including costs associated with public company reporting

requirements. We also anticipate that we will incur costs associated with relatively recently adopted corporate governance requirements, including requirements of the SEC, and the Nasdaq Global Market. We expect these rules and regulations to increase our legal and financial compliance costs and to make some activities more time-consuming and costly. We also expect that these rules and regulations may 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 individuals to serve on our board of directors or as executive officers.

We are currently evaluating and monitoring developments with respect to these rules, and we cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to Section 404 of the Sarbanes-Oxley Act, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting. While we remain an emerging growth company, however, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. We conduct a process each year to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we dedicate internal resources, engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

We have broad discretion over the use of our cash and investments and may not use them effectively.

Our management has broad discretion to use our cash and investments to fund our operations and could spend these funds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending the use of our cash and investments to fund our operations, we may invest these resources in a manner that does not produce income or that loses value.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our share price and trading volume could decline.

The trading market for our common stock may be influenced, in part, by the research and reports that industry or securities analysts publish about us or our business. If no or few securities or industry analysts cover our business, or one or more of the analysts who cover us issues an adverse opinion about our company, our stock price may decline. If one or more of these analysts ceases research coverage of us or fails to regularly publish reports on us, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline. Additionally, if analyst estimates for the commercial value of our product candidates differ materially from the ultimate commercial value of such candidates, the price of our common stock may decline and our ability to raise capital may be impaired.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

(a) Recent Sales of Unregistered Securities

None.

(b) Use of Proceeds from Initial Public Offering

Not applicable.

(c) Issuer Purchases of Equity Securities

None.

ITEM 6. EXHIBITS.

Exhibit Number	Description
3.1	Amended and Restated Certificate of Incorporation of the Registrant (Incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K (File No. 001-38541) filed with the Securities and Exchange Commission on June 25, 2018).
3.2	Amended and Restated By-laws of the Registrant (Incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K (File No. 001-38541) filed with the Securities and Exchange Commission on June 25, 2018).
4.1	Specimen Common Stock Certificate (Incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1/A (File No. 333-225178) filed with the Securities and Exchange Commission on June 8, 2018).
4.2	Second Amended and Restated Investors' Rights Agreement by and among the Registrant and certain of its stockholders dated April 2, 2018 (Incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.1*^	Amendment No. 3, effective as of August 1, 2022, to the Exclusive Research, Development Option and License Agreement by and between the Registrant and Heidelberg Pharma Research GmbH, dated as of March 1, 2018.
31.1*	Certification of Principal Executive Officer pursuant to Rules 13a 14(a) or 15d 14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer pursuant to Rules 13a 14(a) or 15d 14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes Oxley Act of 2002.
32.1**	Certifications of the Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes Oxley Act of 2002.
101SCH*	Inline XBRL Taxonomy Extension Schema Document.
101CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document.
101LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document.
101PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document.
101DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document.
104*	Cover Page Interactive Data File (formatted as Inline XBRL with applicable taxonomy extension information contained in Exhibits 101.)

* Filed herewith.
** Furnished herewith.
^ Portions of this exhibit have been omitted in accordance with the rules of the Securities and Exchange Commission.

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.

MAGENTA THERAPEUTICS, INC.

Date: November 3, 2022

By: /s/ Stephen Mahoney

Stephen Mahoney
Chief Financial and Operating Officer
(Principal Financial and Accounting Officer)

CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY [**], HAS BEEN OMITTED BECAUSE IT IS BOTH NOT MATERIAL AND IS THE TYPE THAT MAGENTA THERAPEUTICS, INC. TREATS AS PRIVATE OR CONFIDENTIAL.

Exhibit 10.1

Amendment No. 3

to the

Exclusive Research, Development Option and License Agreement dated March 1, 2018, as amended by Amendments dated July 4, 2019 and October 30, 2019
(the "Agreement")

by and between

Magenta Therapeutics, Inc.

with principal offices located at 100 Technology Square (5th Floor), Cambridge, MA 02139, USA ("**MAGENTA**"),

and

Heidelberg Pharma Research GmbH
(former Heidelberg Pharma GmbH)

with principal offices located at Gregor-Mendel-Str. 22, D-68526 Ladenburg, Germany ("**HDPR**"),

both also referred to each as a "**Party**" or together as "**Parties**".

WHEREAS, Magenta and HDPR wish to amend certain provisions of the Agreement that relate to each Party's rights to use certain intellectual property rights;

NOW, THEREFORE, in consideration of the mutual covenants and promises contained in this Agreement and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties hereto, intending to be legally bound, do hereby agree in this Amendment as follows:

1. Amendments.

a. The following definitions are hereby added to the end of Section 1 of the Agreement:

"**Amendment No. 3**. The term "Amendment No. 3" means Amendment No. 3 to the Agreement dated as of the Amendment No. 3 Effective Date.

Amendment No. 3 Effective Date. The term "Amendment No. 3 Effective Date" means **August 1, 2022**.

Amendment No. 3 Patent Rights. The term "Amendment No. 3 Patent Rights" means Broad Patent Rights, Research Patent Rights and Silencing Patent Rights. For clarity, Amendment No. 3 Patent Rights are not defined as Improvements.

Broad Patent Rights. The term "Broad Patent Rights" means the Patent Rights listed on Exhibit 1 to Amendment No. 3.

Research Patent Rights. The term "Amendment No. 3 Research Patent Rights" means the Patent Rights listed on Exhibit 2 to Amendment No. 3.

Silencing Patent Rights. The term "Silencing Patent Rights" means the Patent Rights listed on Exhibit 3 to Amendment No. 3.

Silencing Field. The term "Silencing Field" means [**]

b. Section 3.6 is hereby deleted and replaced by the following:

"**3.6 Grant Back Licenses.**

3.6.1. Joint IP Rights. MAGENTA hereby grants to HDPR a worldwide, royalty-free, fully paid-up, perpetual, irrevocable, non-exclusive, freely sublicensable (through multiple tiers) license under MAGENTA's right, title and interest in and to Joint IP Rights for non-clinical research purposes only. In addition, MAGENTA hereby grants to HDPR a worldwide, royalty-free, fully paid-up, perpetual, irrevocable, non-exclusive, sublicensable (through multiple tiers) license under MAGENTA's right, title and interest in and to those Joint IP Rights that are necessary for the discovery, manufacture, development, or commercialization of Amanitin Compounds or other HDPR products containing Amanitin Compounds, to develop, use, make, have made, import, export, sell, offer for sale and otherwise exploit products containing Amanitin, Linkers or Amanitin Toxin Constructs, but excluding products directed at (i) any Exclusive

Research Target during the applicable Target Research Term, or (ii) any Development Target.

3.6.2. Amendment No. 3 Patent Rights.

(a) Research Patent Rights. MAGENTA hereby grants to HDPR a worldwide, royalty-free, fully paid-up, perpetual, irrevocable, non-exclusive, freely sublicensable (through multiple tiers) license under MAGENTA's right, title and interest in and to Research Patent Rights for non-clinical research purposes only.

(b) Broad Patent Rights. MAGENTA hereby grants to HDPR a worldwide, royalty-free, fully paid-up, perpetual, irrevocable, non-exclusive, sublicensable (through multiple tiers) license under MAGENTA's right, title and interest in and to Broad Patent Rights to develop, use, make, have made, import, export, sell, offer for sale and otherwise exploit products containing Amanitin, Linkers or Amanitin Toxin Constructs, but excluding products directed at (i) any Exclusive Research Target during the applicable Target Research Term, or (ii) any Development Target.

(c) Silencing Patent Rights. MAGENTA hereby grants to HDPR a worldwide, royalty-free, fully paid-up, perpetual, irrevocable, non-exclusive, sublicensable (through multiple tiers) license under MAGENTA's right, title and interest in and to Silencing Patent Rights in the Silencing Field in the Territory to develop, use, make, have made, import, export, sell, offer for sale and otherwise exploit products containing Amanitin, Linkers or Amanitin Toxin Constructs, but excluding products directed at (i) any Exclusive Research Target during the applicable Target Research Term, or (ii) any Development Target.

3.6.3. Grant Back License Definition. The licenses set forth in Sections 3.6.1 and 3.6.2 are hereby defined as the "**Grant Back Licenses**". For the avoidance of doubt, HDPR is prohibited from using the Grant Back Licenses for the clinical development or commercialization of Compounds or Products.

3.6.4. HDPR Sublicensing. HDPR will have the right to grant sublicenses, through multiple tiers, under the rights granted to it (i) in Section 3.6.2(a), Section 3.6.2(b) and Section 3.6.2(c), to its Affiliates and to Third Parties; provided that any such sublicenses will be granted pursuant to a written agreement that is consistent with the terms and conditions of this Agreement. If HDPR wants to grant any sublicense to the Patent Rights set forth in Section 3.6.2(b) to a Third Party, it shall first provide Magenta with at least [**] prior written notice and the Parties will then discuss whether to coordinate efforts with respect to possibly granting an exclusive license to such Patent Rights to such Third Party. In addition, HDPR will provide written notice to Magenta of any sublicenses that it grants to any Third Party under Sections 3.6.2(b) or (c)."

d. The following new Section 13.2.4 is hereby added to the Agreement immediately following Section 13.2.3:

"13.2.4 The Parties, will use commercially reasonable efforts to file Patent Rights for Improvements as either MAGENTA IP Improvements or as HDPR IP Improvements to the extent reasonably possible, taking into account the requirements of various jurisdictions. Patent Rights may also be filed as Joint IP Rights if the JPC decides that adequate protection for the Compounds and Products in such jurisdiction cannot be achieved only through the filing of Patent Rights directed at MAGENTA IP Improvements and/or at HDPR IP Improvements. By way of non-limiting example, Patent Rights with respect to Improvements to Amanitin, Linkers, or Amanitin Toxin Constructs would likely be filed as HDPR IP Improvements whereas Patent Rights with respect to Improvements to Products and Compounds against Exclusive Development Targets would likely be Joint IP Rights; provided that in each case taking into account any considerations and possible implications to providing adequate protection in doing so."

e. Section 13.5.1 is hereby deleted and replaced with the following:

"HDPR IP Rights. HDPR shall, at its sole cost and acting in its sole discretion, be responsible for Handling the Patent Rights included in the HDPR IP Rights and HDPR Improvements. HDPR shall promptly provide MAGENTA with copies of any material official correspondence to or from patent offices regarding the Patent Rights included in the HDPR Improvements. HDPR shall provide MAGENTA with a reasonable opportunity to review and comment on material filings relating to such Patent Rights before such filings are submitted to any relevant patent office or governmental authority, and shall give reasonable, good faith, consideration to comments offered by MAGENTA with respect thereto in any final filings submitted by HDPR to any relevant patent office or governmental authority; provided that HDPR shall have the final decision with respect thereto. Without limiting the immediately preceding sentence, HDPR shall provide to MAGENTA (i) any draft new first-patent priority application intended for filing, (ii) the draft of any other new application containing data generated under this Agreement (not including continuations or divisionals, but including continuations-in-part) and/or (iii) additional claims to a PCT application or any national equivalent filing thereof, with respect to (i), (ii) and (iii) to the extent that it constitutes an HDPR Improvement (each a "**New HDPR Application**") for review at least [**] before filing. If such [**] review period cannot be maintained due to upcoming disclosure, as discussed in the JPC according to Section 13.7.2(c) below, the Parties will work together in good faith to review on an expedited basis. Should HDPR intend not to Handle a Patent Right included in the HDPR IP Rights (solely to the extent that such HDPR IP Rights are reasonably necessary or useful for the discovery, manufacture, development or commercialization of Compounds or Products) or HDPR Improvements in a country in the Territory, it shall promptly advise MAGENTA thereof. At the written request of MAGENTA, HDPR shall, at its sole discretion, either continue to Handle such Patent Right at its own cost or, at no cost and no further consideration execute and deliver any documents as may be legally required to permit MAGENTA to Handle any such Patent Right, and MAGENTA may thereafter Handle any such Patent

Right at MAGENTA's own cost (subject to the remainder of this Section 13.5.1) and sole discretion, to the extent that MAGENTA desires to do so. For sake of clarity, HDPR shall use commercially reasonable efforts to maintain any such Patent Right which MAGENTA requested to Handle until responsibility for any such Patent Right has been transferred to MAGENTA (at which point such Patent Rights will be deemed "**MAGENTA Handled Patent Rights**" hereunder). For clarity, such MAGENTA Handled Patent Rights will continue to be owned by HDPR. If any royalties become due from MAGENTA to HDPR under Section 9.4 with respect to Net Sales of any Product in a country that is Covered by any MAGENTA Handled Patent Rights in such country but is not Covered by any other Patent Rights included in the HDPR IP Rights, HDPR Improvements or Joint IP Rights in such country, (i) HDPR will promptly reimburse to MAGENTA all costs and expenses incurred by MAGENTA in connection with the applicable MAGENTA Handled Patent Rights, and (ii) MAGENTA shall pay to HDPR any such royalties in accordance to the terms and conditions of this Agreement."

f. Section 13.5.2 is hereby deleted and replaced by the following:

"Section 13.5.2 MAGENTA IP Rights.

(a) MAGENTA shall, at its sole cost and acting in its sole discretion, Handle all Patent Rights included in the MAGENTA IP Rights, MAGENTA IP Improvements, Joint IP Rights, and Amendment No. 3 Patent Rights; provided that MAGENTA shall provide HDPR with a reasonable opportunity to review and comment on material filings relating to Joint IP Rights and any Amendment No. 3 Patent Rights before such filings are submitted to any relevant patent office or governmental authority and shall give reasonable, good faith, consideration to comments offered by HDPR with respect thereto in any final filings submitted by MAGENTA to any relevant patent office or governmental authority; and further provided that MAGENTA shall have the final decision with respect thereto. Without limiting the immediately preceding sentence, MAGENTA shall provide to the JPC (i) any draft new first-patent priority application intended for filing, (ii) the draft of any other new application containing data generated under this Agreement (not including continuations or divisionals, but including continuations-in-part), and/or (iii) additional claims to a PCT application or any initial national equivalent filing thereof, with respect to (i), (ii) and (iii) to the extent that it constitutes a MAGENTA IP Improvement, a Joint IP Right or an Amendment No. 3 Patent Right (each a "**New Application**") for review at least [**] before filing. If such [**] review period cannot be maintained due to upcoming disclosure, as discussed in the JPC according to Section 13.7.2(c) below, the Parties will work together in good faith to review on an expedited basis. MAGENTA shall promptly provide the JPC with copies of any material official correspondence to or from patent offices regarding the Patent Rights included in the Joint IP Rights or Amendment No. 3 Patent Rights. In case of a violation by MAGENTA of this Section 13.5.2(a), (i) such violation shall constitute a material breach of the Agreement (without limiting or impacting whether a breach of any other provision of the Agreement would constitute a material breach) and (ii)

MAGENTA hereby grants to HDPR and HDPR hereby accepts a worldwide, royalty-free, fully paid-up, perpetual, irrevocable, non-exclusive, freely sublicensable (through multiple tiers) license to any Patent Rights filed or prosecuted in violation of this Section 13.5.2(a). If such breach gives rise to HDPR's ability to terminate all or a part of this Agreement under Section 18.2.1 as a result of such breach, HDPR shall be required to provide written notice of termination to MAGENTA within [**] after giving written notice of such breach to MAGENTA according to Section 18.2.1 if HDPR wants to exercise such termination right; for clarity, if HDPR does not deliver such written notice to MAGENTA within such [**] period, HDPR's right to terminate all or a part of this Agreement as a result of such breach shall expire. The foregoing remedy for a violation by MAGENTA of this Section 13.5.2(a) shall be non-exclusive and without prejudice to any other rights or remedies HDPR may seek against MAGENTA for such violation; nor does such foregoing remedy alter any of MAGENTA's rights to dispute such breach or stay such termination if it elects to do so under Section 18.2.1.

(b) Should MAGENTA decide that it does not desire to Handle a Patent Right included in the Joint IP Rights or any of the Amendment No. 3 Patent Rights in a country in the Territory, it shall promptly advise HDPR thereof. At the written request of HDPR, MAGENTA shall, at no cost and no further consideration execute and deliver any documents as may be legally required to effect the assignment of any such Patent Right in such country to HDPR. HDPR may thereafter Handle any such Patent Right at HDPR's own cost and sole discretion, to the extent that HDPR desires to do so. For sake of clarity, MAGENTA shall use commercially reasonable efforts to maintain any such Patent Right which HDPR requested to Handle until the assignment of any such Patent Right has been effected."

g. The following new Section 13.5.3 is hereby added to the Agreement immediately following Section 13.5.2:

"Section 13.5.3.

(a) Commencing on the Amendment No. 3 Effective Date and continuing for the remainder of the Agreement Term, MAGENTA shall not file any patent application disclosing, or otherwise disclose to a Third Party (except to Permitted Recipients and as required pursuant to Applicable Law or a valid order of a court of competent jurisdiction or governmental body), any Know-How generated by or for MAGENTA in the performance of Technology Research Activities in connection with any Target (all such Know-How, "**Target-Based IP**") unless and until MAGENTA exercises its Exclusive Research Option Right with respect to such Target; for clarity, the Know-How in the previous clause only refers to Know-How generated (i) through the use or application of any Know-How owned or Controlled by HDPR or (ii) using Amanitin, Amanitin Toxin Constructs, Antibody-drug conjugates or Confidential Information provided by HDPR to Magenta. Target-Based IP excludes Know-How that (i) is or becomes generally available to the public other than through fault (whether by action or inaction) or negligence of MAGENTA or its Permitted Recipients, (ii) can be evidenced by

MAGENTA's written records to have been already known to MAGENTA, (iii) is obtained at any time lawfully by MAGENTA from a Third Party under circumstances permitting its use or disclosure, or (iv) is developed independently by or for MAGENTA as evidenced by written records other than through knowledge of Know-How generated by or for MAGENTA in the performance of Technology Research Activities. In case of a violation by MAGENTA of this Section 13.5.3(a), (1) such violation shall constitute a material breach of the Agreement (without limiting or impacting whether a breach of any other provision of the Agreement would constitute a material breach) and (2) MAGENTA hereby grants to HDPR and HDPR hereby accepts a worldwide, royalty-free, fully paid-up, perpetual, irrevocable, non-exclusive, freely sublicensable (through multiple tiers) license under MAGENTA's right, title and interest in and to any Patent Rights filed in violation of this Section 13.5.3(a) to develop, use, make, have made, import, export, sell and offer for sale products containing Amanitin, Linkers or Amanitin Toxin Constructs, but excluding products directed at a Development Target. The foregoing remedy for a violation by MAGENTA of this Section 13.5.3(a) shall be exclusive and HDPR may not seek other retribution against MAGENTA for such violation.

(b) Upon exercise of its Exclusive Research Option Right with respect to a Target, (i) the obligations of non-filing and non-disclosure in Section 13.5.3.(a) shall terminate with respect to the Target-Based IP for such Target; and (ii) the Parties shall have the right to Handle Patent Rights disclosing Target-Based IP for such Target (all such Patent Rights, "**Target-Based Patent Rights**") pursuant to Section 13. If (i) MAGENTA does not exercise the Development Option Right during the applicable Target Research Term or (ii) this Agreement or a license is terminated with respect to a Product, Exclusive Research Target or Development Target by HDPR for MAGENTA's material breach in accordance with Section 18.2.1 or by MAGENTA at-will pursuant to Section 18.2.3, MAGENTA will, and hereby does grant to HDPR, effective upon the lapse of the applicable Target Research Term or termination, respectively, a worldwide, royalty-free, fully paid-up, perpetual, irrevocable, non-exclusive, freely sublicensable (through multiple tiers) license under MAGENTA's rights, titles and interests in and to such Target-Based Patent Rights to develop, use, make, have made, import, export, sell and offer for sale products containing Amanitin, Linkers or Amanitin Toxin Constructs, but excluding products directed at a Development Target."

h. The following new Section 13.7 is hereby added to the Agreement immediately following Section 13.6:

"13.7 **Joint Patent Committee**. Within fifteen (15) Business Days after the Amendment No. 3 Effective Date, the Parties will establish a joint patent committee (the "**Joint Patent Committee**" or "**JPC**"), which shall be independent of the JSC to oversee and coordinate certain Patent Rights and related matters as set forth in this Section 13.

13.7.1 Formation and Composition. The JPC will be comprised of at least one (1) but up to two (2) attorneys representing each

Party. Each Party's representative(s) on the JPC will include the senior attorney of the Party or otherwise have the seniority and experience appropriate in light of the functions and responsibilities of the JPC. In addition, each Party may invite a reasonable number of additional subject matter experts, outside counsel or relevant personnel of such Party to participate in discussions and meetings of the JPC on an ad-hoc basis. Each Party may replace its representative(s) on the JPC at any time by providing notice in writing to the other Party. The JPC will conduct its responsibilities hereunder in good faith and with reasonable care and diligence.

13.7.2 Function and Powers. The JPC will:

(a) in coordination with the JSC, act as a forum for discussion and review of any Improvements;

(b) discuss the delineation of all Patent Rights that contain or constitute Improvements as MAGENTA IP Improvements, HDPR IP Improvements or Joint IP Rights;

(c) discuss any upcoming publications that might trigger the need for the filing of a New HDPR Application or of a New Application;

(d) coordinate and oversee, pursuant to this Section 13, the filing, prosecution, maintenance and enforcement of all Patent Rights that Cover, rely on or are derived from Improvements; and

(e) perform such other functions as set forth in this Agreement, or as the Parties may mutually agree, except where in conflict with any provision of this Agreement.

13.7.3 Meetings, Procedural Rules and Minutes. The JPC shall meet in person, by teleconference or by videoconference at least semi-annually, or with such other frequency as the Parties may mutually agree. Meetings of the JPC will be effective only if at least one (1) JPC representative from each Party is present or participating in such meeting (at which time, a quorum will exist), and each Party shall use reasonable efforts to ensure that at least one (1) of its JPC representatives attends each such meeting. The JPC will take action by consensus of the JPC representatives present at a meeting at which a quorum exists, with each Party having a single vote irrespective of the number of JPC representatives of such Party in attendance, and each Party will use good faith efforts to reach consensus on all issues discussed during the JPC meeting. Unless the Parties agree otherwise, no minutes will be prepared for JPC meetings. Any dispute between the Parties with regard to any matters within the purview of the JPC should be subject to procedures set forth in Section 13.7.4.

13.7.4 Patent Resolution Procedures. In the event the Parties cannot agree on a particular action with respect to any Patent Rights or related matters presented at any JPC meeting or within a period of [**]

thereafter, or other time period that the Parties mutually agree upon, then in each case, such matter will be managed in accordance with Section 19.2."

i. The following new Sections 14.4 and 14.5 are hereby added to the Agreement immediately following Section 14.3:

"14.4 **Representations and Warranties of MAGENTA**. MAGENTA represents and warrants to HDPR that, as of the Amendment No. 3 Effective Date, that:

(a) no Third Party has any right, title or interest in the Amendment No. 3 Patent Rights;

(b) MAGENTA is entitled to grant the licenses under the Amendment No. 3 Patent Rights;

(c) the grant of the licenses by MAGENTA under the Amendment No. 3 Patent Rights does not require MAGENTA to notify any Third Party, or exercise any right, perform any obligation, or otherwise satisfy any requirement under any agreement between MAGENTA and a Third Party that would prevent the grant of such licenses; and

(d) other than the Amendment No. 3 Patent Rights listed in Exhibits 1 through 3 herein, MAGENTA does not own or Control any other Patent Rights that arise or was derived from MAGENTA's use of any (i) Know-How owned or Controlled by HDPR or (ii) Amanitin, Amanitin Toxin Constructs, Antibody-drug conjugates or Confidential Information, in each case, provided by HDPR to MAGENTA; excluding those Patent Rights listed in Exhibit 4.

14.5 **Covenants of MAGENTA**. MAGENTA covenants to HDPR that, from and after the Amendment No. 3 Effective Date and during the Agreement Term of this Agreement:

(a) it shall not, and shall cause its Affiliates to not, assign, transfer, convey, option or grant any rights to the Amendment No. 3 Patent Rights that are inconsistent with or would conflict with or limit the scope of any of the rights or licenses granted to HDPR hereunder; and

(b) it shall not, and shall cause its Affiliates to not (i) license, sell, assign or otherwise transfer to any Person (other than to a successor in interest as permitted under Section 19.3) any Amendment No. 3 Patent Rights (or agree to do any of the foregoing) or (ii) incur or permit to exist, with respect to any Amendment No. 3 Patent Rights, any lien, encumbrance, charge, security interest, mortgage, liability, grant of license to Third Parties or other restriction (including in connection with any indebtedness) that would reduce or adversely affect HDPR's rights hereunder."

j. Section 18.3.2(a) is hereby deleted and replaced by the following:

> "all rights and licenses granted by a Party to another Party under this Agreement shall terminate; provided that MAGENTA's Grant Back License to HDPR under Section 3.6 of this Agreement shall not terminate, but shall continue to remain in full force and effect;"

2. The Parties agree that HDPR will effect a Transfer to MAGENTA, or its designee, of all Know-How Controlled by HDPR or [**], or its successor, as applicable, that is necessary for the process for the manufacture of Amanitin Toxin Construct and Amanitin in non-GMP and GMP-quality, including all associated methods, standards and processes as more fully described in the Manufacturing Technology Transfer Agreement between the Parties effective as of the Amendment No. 3. Effective Date.

3. Release. The Parties, on behalf of themselves and their Affiliates, directors, officers, successors and assigns, releases each other and their respective Affiliates, directors, officers, successors and assigns from any and all claims arising from or related to Section 13 of the Agreement (the "**Released Claims**"), known or unknown, that accrued before the Amendment No. 3 Effective Date. The Parties represent and warrant that they: (i) have the authority to enter into this Release; and (ii) have not assigned the Released Claims, in whole or in part.

4. Controlling Nature; Modification; No Other Changes. Upon the execution of this Amendment by the authorized representative of each Party, the Agreement shall be amended in accordance herewith, and this Amendment shall form a part of the Agreement for all purposes. Except as expressly provided in Section 1 of this Amendment, all other terms as set forth in the Agreement shall remain the same and shall remain in full force and effect.

5. Governing Law. This Amendment shall be governed by the laws of the State of New York, USA, without regard to any conflicts of laws concepts that would apply the substantive law of some other jurisdiction.

6. Counterparts. This Amendment may be executed in counterparts with the same effect as if both Parties had signed the same document. All such counterparts shall be deemed an original, shall be construed together and shall constitute one and the same instrument. Signatures to this Amendment transmitted by email in "portable document format" (".pdf"), or by any other electronic means intended to preserve the original graphic and pictorial appearance of this Amendment shall have the same effect as physical delivery of the paper document bearing original signature.

7. Except as expressly set forth in this Amendment, all other provisions of the Agreement shall remain unaffected and in full force and effect.

[*Signature pages follow*]

IN WITNESS WHEREOF, the Parties have entered into this Amendment No. 3 to the Agreement as of the Amendment No. 3 Effective Date.

HEIDELBERG PHARMA RESEARCH GMBH

By: */s/ Prof. Dr. Andreas Pahl*

Name: Prof. Dr. Andreas Pahl

Title: CSO

By: */s/ Dr. George Badescu*

Name: Dr. George Badescu

Title: CBO

MAGENTA THERAPEUTICS, INC.

By: */s/ Jason Gardner*

Name: Jason Gardner

Title: President and CEO

Broad Patent Rights

[**]

Exhibit 2

Research Patent Rights

[**]

Exhibit 3

Silencing Patent Rights

[**]

14

Excluded Patent Rights

[**]

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER PURSUANT TO RULE 13A-14(A) / RULE 15D-14(A) OF THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED

I, Jason Gardner, D.Phil., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Magenta Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer 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 the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 3, 2022

/s/ Jason Gardner

Jason Gardner, D.Phil.
President, Chief Executive Officer and Director
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER PURSUANT TO RULE 13A-14(A) / RULE 15D-14(A) OF THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED

I, Stephen Mahoney, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Magenta Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer 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 the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 3, 2022

/s/ Stephen Mahoney

Stephen Mahoney
Chief Financial and Operating Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATIONS OF PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with this Quarterly Report on Form 10-Q of Magenta Therapeutics, Inc. (the "Company") for the quarter ended September 30, 2022 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of his or her 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.

Dated: November 3, 2022

/s/ Jason Gardner

Jason Gardner, D.Phil.
President, Chief Executive Officer and Director
(Principal Executive Officer)

/s/ Stephen Mahoney

Stephen Mahoney
Chief Financial and Operating Officer
(Principal Financial and Accounting Officer)