

---

---

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, DC 20549**

---

**FORM 10-Q**

---

(Mark One)

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

For the quarterly period ended June 30, 2019

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: 001-38550

---

**Translate Bio, Inc.**

(Exact Name of Registrant as Specified in its Charter)

---

**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**61-1807780**  
(I.R.S. Employer  
Identification No.)

**29 Hartwell Avenue**  
**Lexington, Massachusetts**  
(Address of principal executive offices)

**02421**  
(Zip Code)

Registrant's telephone number, including area code: (617) 945-7361

---

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	TBIO	Nasdaq Global Select Market

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

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

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

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

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

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

As of July 29, 2019, the registrant had 51,010,368 shares of common stock, \$0.001 par value per share, outstanding.

---

---

**Table of Contents**

	<u>Page</u>
<b>PART I. FINANCIAL INFORMATION</b>	1
Item 1. <a href="#">Financial Statements (Unaudited)</a>	1
<a href="#">Condensed Consolidated Balance Sheets as of June 30, 2019 and December 31, 2018</a>	1
<a href="#">Condensed Consolidated Statements of Operation for the Three and Six Months Ended June 30, 2019 and 2018</a>	2
<a href="#">Condensed Consolidated Statements of Comprehensive Loss for the Three and Six Months Ended June 30, 2019 and 2018</a>	3
<a href="#">Condensed Consolidated Statements of Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit) for the Three and Six Months Ended June 30, 2019 and 2018</a>	4
<a href="#">Condensed Consolidated Statements of Cash Flows for the Six Months Ended June 30, 2019 and 2018</a>	5
<a href="#">Notes to Condensed Consolidated Financial Statements (Unaudited)</a>	6
Item 2. <a href="#">Management's Discussion and Analysis of Financial Condition and Results of Operations</a>	24
Item 3. <a href="#">Quantitative and Qualitative Disclosures About Market Risk</a>	39
Item 4. <a href="#">Controls and Procedures</a>	39
<b>PART II. OTHER INFORMATION</b>	41
Item 1. <a href="#">Legal Proceedings</a>	41
Item 1A. <a href="#">Risk Factors</a>	41
Item 2. <a href="#">Unregistered Sales of Equity Securities and Use of Proceeds</a>	84
Item 6. <a href="#">Exhibits</a>	85
<a href="#">Signatures</a>	86

**PART I—FINANCIAL INFORMATION**

**Item 1. Financial Statements.**

**TRANSLATE BIO, INC.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS (UNAUDITED)**  
(In thousands, except share and per share amounts)

	June 30, 2019	December 31, 2018
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 74,904	\$ 55,199
Short-term investments	71,961	88,904
Prepaid expenses and other current assets	6,069	4,474
Restricted cash	950	1,025
Total current assets	153,884	149,602
Property and equipment, net	10,903	10,245
Right-of-use assets, net	10,650	—
Goodwill	21,359	21,359
Intangible assets, net	105,630	106,445
Deferred offering costs	123	—
Total assets	<u>\$ 302,549</u>	<u>\$ 287,651</u>
<b>Liabilities and Stockholders' Equity (Deficit)</b>		
Current liabilities:		
Accounts payable	\$ 3,347	\$ 5,168
Accrued expenses	7,246	6,547
Current portion of deferred revenue	9,015	2,572
Current portion of operating lease liability	449	—
Total current liabilities	20,057	14,287
Long-term portion of contingent consideration	120,233	103,642
Deferred revenue, net of current portion	34,192	41,841
Deferred tax liabilities	—	481
Deferred rent	—	2,105
Operating lease liability, net of current portion	12,370	—
Total liabilities	186,852	162,356
Commitments and contingencies (Notes 3, 4 and 13)		
Stockholders' equity (deficit):		
Preferred stock, \$0.001 par value; 10,000,000 shares authorized as of June 30, 2019 and December 31, 2018; no shares issued and outstanding as of June 30, 2019 and December 31, 2018	—	—
Common stock, \$0.001 par value; 200,000,000 shares authorized as of June 30, 2019 and December 31, 2018; 51,010,368 shares and 45,139,955 shares issued and outstanding as of June 30, 2019 and December 31, 2018, respectively	51	45
Additional paid-in capital	422,309	371,257
Accumulated deficit	(307,233)	(246,203)
Accumulated other comprehensive income	570	196
Total stockholders' equity (deficit)	115,697	125,295
Total liabilities and stockholders' equity (deficit)	<u>\$ 302,549</u>	<u>\$ 287,651</u>

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

**TRANSLATE BIO, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (UNAUDITED)**  
**(In thousands, except share and per share amounts)**

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2019</u>	<u>2018</u>	<u>2019</u>	<u>2018</u>
Collaboration revenue	\$ 1,174	\$ —	\$ 2,648	\$ —
Operating expenses:				
Research and development	16,625	15,219	34,048	27,921
General and administrative	7,850	5,991	14,403	10,769
Change in fair value of contingent consideration	4,889	7,852	16,591	12,760
Total operating expenses	29,364	29,062	65,042	51,450
Loss from operations	(28,190)	(29,062)	(62,394)	(51,450)
Other income (expense):				
Interest income	358	91	878	181
Other expense	—	(32)	—	(45)
Total other income (expense), net	358	59	878	136
Loss before benefit from income taxes	(27,832)	(29,003)	(61,516)	(51,314)
Benefit from income taxes	—	1,500	486	2,602
Net loss	(27,832)	(27,503)	(61,030)	(48,712)
Accretion of redeemable convertible preferred stock to redemption value	—	(459)	—	(644)
Net loss attributable to common stockholders	\$ (27,832)	\$ (27,962)	\$ (61,030)	\$ (49,356)
Net loss per share attributable to common stockholders—basic and diluted	\$ (0.57)	\$ (3.04)	\$ (1.30)	\$ (5.40)
Weighted average common shares outstanding—basic and diluted	48,749,627	9,187,207	46,866,842	9,139,638

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

**TRANSLATE BIO, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS (UNAUDITED)**  
**(In thousands)**

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2019</u>	<u>2018</u>	<u>2019</u>	<u>2018</u>
Net loss	\$ (27,832)	\$ (27,503)	\$ (61,030)	\$ (48,712)
Other comprehensive income (loss):				
Unrealized gains (losses) on available-for-sale securities, net of tax of \$0	219	—	374	(79)
Comprehensive loss	<u>\$ (27,613)</u>	<u>\$ (27,503)</u>	<u>\$ (60,656)</u>	<u>\$ (48,791)</u>

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

**TRANSLATE BIO, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK**  
**AND STOCKHOLDERS' EQUITY (DEFICIT) (UNAUDITED)**  
(In thousands, except share amounts)

	Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
<b>Balances at December 31, 2018</b>	—	\$ —	45,139,955	\$ 45	\$ 371,257	\$ (246,203)	\$ 196	\$ 125,295
Exercise of stock options	—	—	154,484	—	897	—	—	897
Stock-based compensation expense	—	—	—	—	1,959	—	—	1,959
Unrealized gains on available-for-sale securities	—	—	—	—	—	—	155	155
Net loss	—	—	—	—	—	(33,198)	—	(33,198)
<b>Balances at March 31, 2019</b>	—	—	45,294,439	45	374,113	(279,401)	351	95,108
Issuance of common stock in connection with private placement, net of placement agent fees and offering costs	—	—	5,582,940	6	44,128	—	—	44,134
Issuance of common stock in connection with a former employee letter agreement (Note 9)	—	—	67,406	—	847	—	—	847
Forfeited restricted common stock	—	—	(1,334)	—	(1)	—	—	(1)
Exercise of stock options	—	—	66,917	—	519	—	—	519
Stock-based compensation expense	—	—	—	—	2,703	—	—	2,703
Unrealized gains on available-for-sale securities	—	—	—	—	—	—	219	219
Net loss	—	—	—	—	—	(27,832)	—	(27,832)
<b>Balances at June 30, 2019</b>	—	\$ —	51,010,368	\$ 51	\$ 422,309	\$ (307,233)	\$ 570	\$ 115,697

	Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
<b>Balances at December 31, 2017</b>	142,288,292	\$192,896	9,582,791	\$ 10	\$ 55,204	\$ (148,808)	\$ 79	\$ (93,515)
Accretion of redeemable convertible preferred stock to redemption value	—	185	—	—	(185)	—	—	(185)
Stock-based compensation expense	—	—	—	—	1,383	—	—	1,383
Unrealized losses on available-for-sale securities	—	—	—	—	—	—	(79)	(79)
Net loss	—	—	—	—	—	(21,209)	—	(21,209)
<b>Balances at March 31, 2018</b>	142,288,292	193,081	9,582,791	10	56,402	(170,017)	—	(113,605)
Accretion of redeemable convertible preferred stock to redemption value	—	459	—	—	(459)	—	—	(459)
Forfeited restricted common stock	—	—	(311)	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	2,875	—	—	2,875
Exercise of stock options	—	—	49,459	—	278	—	—	278
Net loss	—	—	—	—	—	(27,503)	—	(27,503)
<b>Balances at June 30, 2018</b>	142,288,292	193,540	9,631,939	10	59,096	(197,520)	—	(138,414)

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

**TRANSLATE BIO, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS (UNAUDITED)**  
(In thousands)

	<u>Six Months Ended June 30,</u>	
	<u>2019</u>	<u>2018</u>
<b>Cash flows from operating activities:</b>		
Net loss	\$ (61,030)	\$ (48,712)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization expense	1,960	1,447
Stock-based compensation expense	5,509	4,258
Change in fair value of contingent consideration	16,591	12,760
Deferred income tax benefit	(486)	(2,602)
Accretion of discount on short-term investments	—	43
Changes in operating assets and liabilities, net of effects of acquisition:		
Prepaid expenses and other assets	(1,766)	(2,509)
Right-of-use assets	234	—
Accounts payable	(1,858)	1,081
Accrued expenses	600	266
Deferred rent	—	387
Lease liability	(170)	—
Deferred revenue	(1,029)	—
Net cash used in operating activities	<u>(41,445)</u>	<u>(33,581)</u>
<b>Cash flows from investing activities:</b>		
Purchases of investments	(38,438)	(6,000)
Sales and maturities of investments	55,756	14,918
Purchases of property and equipment	(1,793)	(4,757)
Net cash provided by investing activities	<u>15,525</u>	<u>4,161</u>
<b>Cash flows from financing activities:</b>		
Proceeds from private placement, net of placement agent fees	44,608	—
Payments of private placement offering costs	(474)	—
Payments of initial public offering costs	—	(2,421)
Proceeds from option exercises	1,416	278
Net cash provided by (used in) financing activities	<u>45,550</u>	<u>(2,143)</u>
<b>Net increase (decrease) in cash, cash equivalents and restricted cash</b>	<u>19,630</u>	<u>(31,563)</u>
Cash, cash equivalents and restricted cash at beginning of period	56,224	50,024
Cash, cash equivalents and restricted cash at end of period	<u>\$ 75,854</u>	<u>\$ 18,461</u>
<b>Cash, cash equivalents and restricted cash at end of period:</b>		
Cash and cash equivalents	\$ 74,904	\$ 16,495
Restricted cash	950	1,966
Total cash, cash equivalents and restricted cash at end of period	<u>\$ 75,854</u>	<u>\$ 18,461</u>
<b>Supplemental disclosure of non-cash investing and financing activities:</b>		
Purchases of property and equipment included in accounts payable and accrued expenses	\$ 59	\$ 489
Deferred offering costs included in accounts payable and accrued expenses	\$ 123	\$ 1,402
Issuance of common stock in connection with a former employee letter agreement (Note 9)	\$ 847	\$ —
Accretion of redeemable convertible preferred stock to redemption value	\$ —	\$ 644

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

**TRANSLATE BIO, INC.**  
**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS**  
**(UNAUDITED)**

**1. Nature of the Business and Basis of Presentation**

Translate Bio, Inc. (the “Company”) is a clinical-stage messenger RNA (“mRNA”) therapeutics company developing a new class of potentially transformative medicines to treat diseases caused by protein or gene dysfunction. Using its proprietary mRNA therapeutic platform (“MRT platform”), the Company creates mRNA that encodes functional proteins. The Company’s mRNA is delivered to the target cell where the cell’s own machinery recognizes it and translates it, restoring or augmenting protein function to treat or prevent disease. The Company is initially focused on restoring the expression of intracellular and transmembrane proteins, areas that have eluded conventional protein therapeutics, in patients with genetic diseases where there is high unmet medical need.

The Company is developing its lead MRT product candidate for the lung, MRT5005, for the treatment of cystic fibrosis (“CF”). The Company is conducting a Phase 1/2 clinical trial to evaluate the safety and tolerability of single and multiple-ascending doses of MRT5005. Percent predicted forced expiratory volume in one second (“ppFEV1”), which is a well-defined and accepted endpoint measuring lung function, is also being measured at pre-defined timepoints throughout the trial. In April 2019, the Company completed dosing of all patients in the single-ascending dose (“SAD”) portion of the Phase 1/2 clinical trial and on July 31, 2019, the Company reported interim data from the SAD portion of the clinical trial. MRT5005 was generally well tolerated at low and mid-dose levels with no serious adverse events reported at any dose level. Marked increases in ppFEV1 were observed after a single dose of MRT5005, primarily at mid-dose. Based on the analysis of the interim results, the Company plans to amend the clinical trial protocol to include two additional cohorts of patients. In early 2019, the Company began dosing patients in the multiple-ascending dose (“MAD”) portion of this trial and expects to report results from the additional SAD cohort and the ongoing MAD portion of the clinical trial in 2020.

The Company is developing its lead MRT product candidate for the liver, MRT5201, for the treatment of ornithine transcarbamylase (“OTC”) deficiency. In June 2019, the Company received clearance from the U.S. Food and Drug Administration (the “FDA”) to proceed with a SAD Phase 1/2 clinical trial of MRT5201 in adult patients with OTC deficiency. In December 2018, the Company had submitted an investigational new drug application (“IND”) for a SAD and MAD Phase 1/2 clinical trial of MRT5201, which the FDA placed on clinical hold, pending additional preclinical toxicology data. After discussions with the FDA and after the Company amended the protocol, the FDA removed the clinical hold and allowed the Company to move forward with a SAD clinical trial. The Company plans to initiate patient screening for the SAD Phase 1/2 clinical trial in the second half of 2019. The Company is currently conducting additional preclinical studies that are required to support future clinical development of MRT5201, including a MAD clinical trial, and plans to submit data from these preclinical studies to the FDA in the fourth quarter of 2019.

The Company is subject to risks 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 and the ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including 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 product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

The accompanying condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America (“GAAP”) and include the accounts of the Company and its two wholly owned subsidiaries, Translate Bio MA, Inc. and Translate Bio Securities Corporation, from their date of incorporation. All intercompany accounts and transactions have been eliminated in consolidation.

---

## [Table of Contents](#)

The accompanying unaudited condensed consolidated balance sheet as of June 30, 2019, the unaudited condensed consolidated statements of operations and of comprehensive loss for the three and six months ended June 30, 2019 and 2018, the unaudited condensed consolidated statements of redeemable convertible preferred stock and stockholders' equity (deficit) for the three and six months ended June 30, 2019 and 2018 and of the unaudited condensed consolidated statements of cash flows for the six months ended June 30, 2019 and 2018 have been prepared by the Company pursuant to the rules and regulations of the Securities and Exchange Commission ("SEC") for interim financial statements. The accompanying balance sheet as of December 31, 2018 has been derived from the Company's audited financial statements for the year ended December 31, 2018 previously filed with the SEC. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to rules and regulations. However, the Company believes that the disclosures are adequate to make the information presented not misleading. The accompanying unaudited interim consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements and the notes thereto for the year ended December 31, 2018 included in the Company's Annual Report on Form 10-K that was filed with the SEC on March 21, 2019.

The accompanying unaudited interim condensed consolidated financial presentation has been prepared on the same basis as the audited annual consolidated financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's financial position as of June 30, 2019, the results of its operations for the three and six months ended June 30, 2019 and 2018, and its cash flows for the six months ended June 30, 2019 and 2018. The financial data and other information disclosed in these notes related to the three and six months ended June 30, 2019 and 2018 are also unaudited. The results for the three and six months ended June 30, 2019 are not necessarily indicative of results to be expected for the year ending December 31, 2019, any other interim periods, or any future year or period.

### ***Acquisition of Shire's MRT Program***

In December 2016, the Company entered into an asset purchase agreement (as amended in June 2018, the "Shire Agreement") with Shire Human Genetic Therapies, Inc. ("Shire"), a subsidiary of Takeda Pharmaceutical Company Ltd., pursuant to which Shire sold equipment to and assigned to the Company all of its rights to certain patent rights, permits, real property leases, contracts, regulatory documentation, books and records, and materials related to Shire's mRNA therapy platform (the "MRT Program"), including its cystic fibrosis transmembrane conductance regulator ("CFTR") and OTC deficiency mRNA therapeutic programs.

### ***Reverse Stock Split***

On June 15, 2018, the Company effected a one-for-5.5555 reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each series of the Company's redeemable convertible preferred stock (see Note 8). Accordingly, all share and per share amounts for all periods presented in the accompanying condensed consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect this reverse stock split and the associated adjustment of the preferred stock conversion ratios.

### ***Sales of Common Stock***

On July 2, 2018, the Company closed its initial public offering of its common stock (the "IPO"). In the IPO, the Company issued and sold 9,714,371 shares of common stock, including the underwriters' over-allotment option, at a public offering price of \$13.00 per share, resulting in aggregate net proceeds of \$113.2 million after deducting underwriting discounts and commissions and offering expenses.

On May 3, 2019, the Company issued and sold 5,582,940 shares of its common stock in a private placement at a price per share of \$8.50, resulting in gross proceeds of \$47.5 million, before deducting placement agent fees of \$2.8 million and other offering expenses of \$0.6 million.

### ***Sanofi Pasteur Collaboration and Licensing Agreement***

In 2018, the Company entered into a collaboration and license agreement with Sanofi Pasteur Inc. ("Sanofi"), the vaccines global business unit of Sanofi S.A., to develop mRNA vaccines for up to five infectious disease pathogens (the "Sanofi Agreement"). Under the Sanofi Agreement, the Company and Sanofi are jointly conducting research and development activities to advance mRNA vaccines and mRNA vaccine platform development during a three-year research term, which may be extended by mutual agreement. Following the research term, the Company is obligated to manufacture clinical product for Sanofi, which the Company estimates may take up to eight years to complete.

---

## [Table of Contents](#)

The Company is eligible to receive up to \$805.0 million in payments, including an upfront payment of \$45.0 million, which the Company received in 2018; certain development, regulatory and sales-related milestones across several vaccine targets; and option exercise fees if Sanofi exercises its option related to development of vaccines for additional pathogens. The Company is also eligible to receive reimbursable development costs and tiered royalty payments associated with worldwide sales of the developed vaccines, if any (see Note 3).

### ***Going Concern***

In accordance with Accounting Standards Update (“ASU”) No. 2014-15, *Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern (Subtopic 205-40)*, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company’s ability to continue as a going concern within one year after the date that the consolidated financial statements are issued.

The Company’s financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. Through June 30, 2019, the Company has funded its operations with proceeds from the sale of redeemable convertible preferred stock and the sale of bridge units, which ultimately converted into shares of the Company’s common stock, the proceeds from the IPO, the upfront payment received under the Sanofi Agreement and the proceeds from a private placement of the Company’s common stock. The Company has incurred recurring losses and cash outflows from operations since its inception, including net losses of \$61.0 million and \$48.7 million for the six months ended June 30, 2019 and 2018, respectively. In addition, the Company had an accumulated deficit of \$307.2 million as of June 30, 2019. The Company expects to continue to generate operating losses for the foreseeable future.

As of July 31, 2019, the date of issuance of these unaudited interim condensed consolidated financial statements, the Company expects that its cash, cash equivalents and short-term investments of \$146.9 million as of June 30, 2019 will be sufficient to fund its operating expenses and capital expenditure requirements into the second half of 2020. The future viability of the Company beyond that point is dependent on the Company’s ability to raise additional capital to finance its operations.

Although the Company has been successful in raising capital in the past, there is no assurance that it will be successful in obtaining such additional financing on terms acceptable to the Company, if at all. The Company expects that its expenses will increase in connection with its ongoing business activities. As a result, the Company will need substantial additional funding to support its continuing operations and pursue its growth strategy. Until such time as the Company can generate significant revenue from product sales, if ever, it expects to finance its operations through the sale of equity, debt financings or other capital sources, including collaborations with other companies or other strategic transactions. The Company may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If the Company is unable to obtain funding, the Company will be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or commercialization efforts, which could adversely affect its business prospects, or the Company may be unable to continue operations.

Based on its recurring losses and cash outflows from operations since inception, expectation of continuing operating losses and cash outflows from operations for the foreseeable future and the need to raise additional capital to finance its future operations, the Company concluded that there was substantial doubt about its ability to continue as a going concern.

## **2. Summary of Significant Accounting Policies**

The significant accounting policies and estimates used in preparation of the consolidated financial statements are described in the Company’s audited financial statements as of and for the year ended December 31, 2018, and the notes thereto, which are included in the Company’s Annual Report on Form 10-K. During the six months ended June 30, 2019, there were no material changes to the Company’s significant accounting policies, except for the adoption of ASU No. 2016-02, *Leases (Topic 842)* (“ASU 2016-02”), as described more fully under the heading “Recently Adopted Accounting Pronouncements”.

### ***Recently Adopted Accounting Pronouncements***

In February 2016, the Financial Accounting Standards Board (the “FASB”) issued ASU 2016-02, which requires lessees to recognize most leases on their balance sheet as a right-of-use (“ROU”) asset and a lease liability. The Company adopted ASU 2016-02 as of the required effective date of January 1, 2019 using the cumulative-effect adjustment on the effective date of the standard, with comparative periods presented in accordance with the previous guidance in ASC 840. Subsequent to the issuance of Topic 842, the FASB clarified the guidance through several ASUs; hereinafter the collection of lease guidance is referred to as “ASC 842”.

---

## [Table of Contents](#)

The Company elected the permitted practical expedients within ASC 842, which allowed the Company to not reassess previous accounting conclusions around whether arrangements are or contain leases, and carried forward both the historical classification of leases and the treatment of initial direct costs. In addition, the Company elected to exclude leases with an initial term of one year or less in the recognized ROU assets and lease liabilities.

Adoption of the new standard resulted in the recording of ROU assets and related lease liabilities of approximately \$10.9 million and \$13.0 million, respectively, as of January 1, 2019. The standard did not materially impact the Company's consolidated net earnings and had no impact on cash flows. Refer to Note 12 for the additional disclosures required by ASC 842.

The Company determines if an arrangement is a lease at inception. For leases where the Company is the lessee, ROU assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent an obligation to make lease payments arising from the lease. ROU assets and lease liabilities are recognized at the lease commencement date based on the present value of lease payments over the lease term. As most of the Company's leases do not provide an implicit interest rate, the Company uses its incremental borrowing rate, which are the rates incurred to borrow on a collateralized basis over a term equal to the lease payments in a similar economic environment, in determining the present value of lease payments. The lease terms used to calculate the ROU asset and related lease liability include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Lease expense for operating leases is recognized on a straight-line basis over the lease term as an operating expense. The Company has lease agreements which require payments for lease and non-lease components and has elected to account for these as a single lease component.

In July 2017, the FASB issued ASU No. 2017-11, *Earnings Per Share (Topic 260), Distinguishing Liabilities from Equity (Topic 480), Derivatives and Hedging (Topic 815) I. Accounting for Certain Financial Instruments with Down Round Features II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception* ("ASU 2017-11"). Part I applies to entities that issue financial instruments, such as warrants, convertible debt or convertible preferred stock that contain down-round features. Part II replaces the indefinite deferral for certain mandatorily redeemable noncontrolling interests and mandatorily redeemable financial instruments of nonpublic entities contained within ASC Topic 480 with a scope exception and does not impact the accounting for these mandatorily redeemable instruments. The Company adopted ASU 2017-11 as of the required effective date for annual periods beginning after December 15, 2018, and its adoption had no impact on the Company's financial position, results of operations or cash flows.

In June 2018, the FASB issued ASU No. 2018-07, *Compensation-Stock Compensation (Topic 718)* ("ASU 2018-07"), which aligns the accounting for share-based payment awards issued to employees and non-employees. Under the new guidance, the existing employee guidance will apply to non-employee share-based transactions. The Company adopted ASU 2018-07 as of the required effective date on January 1, 2019. Upon adoption, the Company remeasured the fair value of a grant previously awarded to a non-employee which did not have a material impact on the Company's financial position, results of operations or cash flows.

### **Recently Issued Accounting Pronouncements**

In June 2016, the FASB issued guidance on the Measurement of Credit Losses on Financial Instruments. The guidance requires that credit losses be reported using an expected losses model rather than the incurred losses model that is currently used, and establishes additional disclosures related to credit risks. For available-for-sale debt securities with unrealized losses, the standard now requires allowances to be recorded instead of reducing the amortized cost of the investment. This standard will be effective for the Company on January 1, 2020. The Company does not expect that the adoption of this new standard will have a material impact on its disclosures.

In January 2017, the FASB issued ASU No. 2017-04, *Intangibles—Goodwill and Other: Simplifying the Test for Goodwill Impairment (Topic 350)* ("ASU 2017-04"), which provides for the elimination of Step 2 from the goodwill impairment test. If impairment charges are recognized, the amount recorded will be the amount by which the carrying amount exceeds the reporting unit's fair value with certain limitations. ASU 2017-04 is effective for fiscal years beginning after December 15, 2019. The Company does not expect that the adoption of this new standard will have a material impact on its disclosures.

In August 2018, the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement*. This new standard modifies certain disclosure requirements on fair value measurements. This new standard will be effective on January 1, 2020. Early adoption, of the entire amendments or on the provisions that eliminate or modify the requirements, is permitted. The Company does not expect that the adoption of this new standard will have a material impact on its disclosures.

---

## [Table of Contents](#)

In November 2018, the FASB issued ASU No. 2018-18, *Collaborative Arrangements (Topic 808)* (“ASU 2018-18”). This update provides clarification on the interaction between *Revenue Recognition* (Topic 606) and *Collaborative Arrangements* (Topic 808) including the alignment of unit of account guidance between the two topics. This update is effective in fiscal years, including interim periods, beginning after December 15, 2020, and early adoption is permitted. The Company is currently evaluating the potential impact that the adoption of ASU 2018-18 may have on its consolidated financial statements.

### **3. Sanofi Collaboration and License Agreement**

In 2018, the Company entered into the Sanofi Agreement, a collaboration and license agreement with Sanofi to develop mRNA vaccines and mRNA vaccine platform development for up to five infectious disease pathogens (the “Licensed Fields”).

Under the Sanofi Agreement, the Company and Sanofi have agreed to collaborate to perform certain research and development activities to advance mRNA vaccines and mRNA vaccine platform development during a three-year research term, which may be extended by mutual agreement. Following the research term, the Company is obligated to manufacture clinical product for Sanofi, which the Company estimates may take up to eight years to complete. The collaboration activities will be subject to a collaboration plan to be updated annually. Under the terms of the Sanofi Agreement, the Company received an upfront payment of \$45.0 million and is eligible for certain potential milestone and option payments, each as further described below. In addition, the Company is eligible to receive from Sanofi tiered royalty payments on worldwide net sales of mRNA vaccines.

Under the Sanofi Agreement, the Company and Sanofi created a governance structure, including committees and working groups, to manage the activities under the collaboration. If the Company and Sanofi do not mutually agree on certain decisions, Sanofi would be able to break a deadlock without the Company’s consent. The collaboration includes an estimated budget. Sanofi is responsible for paying reimbursable development costs, including the Company’s employee costs, out-of-pocket costs paid to third parties and manufacturing costs, up to a specified amount. Any reimbursable development costs are payable by Sanofi within 60 days of invoicing.

Under the terms of the Sanofi Agreement, the Company has granted to Sanofi exclusive, worldwide licenses under applicable patents, patent applications, know-how and materials, including those arising under the collaboration, to develop, commercialize and manufacture mRNA vaccines to prevent, treat or cure diseases, disorders or conditions in humans caused by any of three of the Licensed Fields. In addition, pursuant to the terms of the Sanofi Agreement and subject to certain limitations, Sanofi has options to add up to two additional infectious disease pathogens within the granted licenses to the Licensed Fields by exercising either option or both options during a specified option term and paying the Company a \$5.0 million fee per added pathogen. If, prior to the exercise of the options by Sanofi, the Company receives a bona fide third-party offer to acquire rights to the field to which an option relates, the Company must notify Sanofi of such offer, and if Sanofi does not exercise its option as to the applicable field, such field will no longer be subject to the option.

The Company and Sanofi retain the rights to perform their respective obligations and exercise their respective rights under the Sanofi Agreement, and Sanofi may grant sublicenses to affiliates or third parties. Sanofi has also granted the Company non-exclusive, sublicensable licenses under patent rights claiming certain improvements that Sanofi may make to the technology the Company has licensed to it or claiming certain technology arising from the collaboration and owned by Sanofi. The Company may exercise such licenses to develop, manufacture and commercialize products, other than products that use a vaccine to prevent, treat or cure a disease, disorder or condition in humans caused by an infectious disease pathogen. If the Company commercializes any product covered by such a Sanofi patent right, the Company would pay Sanofi a royalty of a low single-digit percentage. Sanofi may terminate these licenses to the Company if the Company materially breaches the terms of the license and the breach remains uncured for a specified period, which may be extended in certain circumstances.

Sanofi has sole responsibility for all commercialization activities for mRNA vaccines in the Licensed Fields and is obligated to bear all costs in connection with any such commercialization. The Company and Sanofi intend to enter into a supply agreement pursuant to which the Company would be responsible for manufacturing certain non-clinical and clinical mRNA vaccines and materials containing mRNA until the Company transfers such manufacturing capabilities to Sanofi. The Company would be entitled to receive payments for manufacturing mRNA vaccines under the supply agreement.

The Sanofi Agreement provides that the Company is eligible to receive aggregate potential payments of up to \$805.0 million from Sanofi, which includes an upfront payment, potential milestone payments and potential option exercise payments. In 2018, Sanofi paid the Company a \$45.0 million upfront payment in respect of the licenses and options granted to Sanofi. Sanofi will also pay the Company \$5.0 million with respect to each additional Licensed Field for which it exercises an option. Sanofi has also agreed to pay the Company milestone payments upon the achievement of specified development, regulatory and commercialization milestones. In particular, the Company is entitled to receive development and regulatory milestone payments of up to \$63.0 million per Licensed Field and sales milestone payments of up to \$85.0 million per Licensed Field. In addition, the Company is entitled to receive a \$10.0 million milestone payment from Sanofi following completion of the technology and process transfer.

---

## [Table of Contents](#)

Sanofi has agreed to pay the Company a tiered royalty on worldwide net sales of all mRNA vaccines within each Licensed Field ranging from a high single-digit percentage to a low teens percentage, depending on quarterly net sales by Sanofi, its affiliates and its sublicensees. The royalty paid to the Company can be reduced with respect to a product once the relevant licensed patent rights expire or if additional licensed technology is required, but the royalty payments generally may not fall below the Company's royalty obligations to third parties plus a royalty of a low single-digit percentage. Royalty payments under the Sanofi Agreement are payable on a product-by-product and country-by-country basis beginning on the launch of the product in the country until the later of the expiration of the last valid claim covering such product or 10 years after the launch of such product in such country.

The Sanofi Agreement provides that it will remain in effect until terminated in accordance with its terms. Either the Company or Sanofi may terminate the Sanofi Agreement in its entirety if the other party is subject to certain insolvency proceedings. Either party may terminate the Sanofi Agreement in its entirety or with respect to a particular Licensed Field, country or product if the other party materially breaches the Sanofi Agreement and the breach remains uncured for a specified period, which may be extended in certain circumstances. Sanofi may also terminate the Sanofi Agreement in its entirety or with respect to a particular Licensed Field, country or product for safety reasons or for convenience, in each case after a specified notice period. After termination of the Sanofi Agreement, Sanofi may continue to manufacture and commercialize the terminated products for a specified period of time, subject to Sanofi's payment obligations.

### ***Accounting Under ASC 606***

In determining the appropriate amount of revenue to be recognized under Accounting Standard Codification ("ASC") 606, *Revenue from Contracts with Customers* ("ASC 606"), the Company performed the following steps: (i) identified the promised goods or services in the contract; (ii) determined whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation.

The Company identified the following promised goods or services contained in the Sanofi Agreement: (i) the license it conveyed to Sanofi with respect to the Licensed Fields, (ii) the licensed know-how to be conveyed to Sanofi with respect to the Licensed Fields, (iii) its obligation to perform research and development on the Licensed Fields, (iv) its obligation to transfer licensed materials to Sanofi, (v) its obligation to manufacture and supply certain non-clinical and clinical mRNA vaccines and materials containing mRNA until the Company transfers such manufacturing capabilities to Sanofi and (vi) the technology and process transfer. The Company assessed whether each of these promised goods or services are distinct performance obligations on their own or if they need to be combined with other promises to create a bundle that is a distinct performance obligation. The Company determined that the promised goods and services do not have standalone value and are highly interrelated. Accordingly, the promised goods and services represent one performance obligation. Sanofi's right to exercise options for up to two additional infectious disease pathogens within the granted licenses to the Licensed Fields are accounted for separately as they do not represent material rights, based on the criteria of ASC 606. Upon the exercise of any option by Sanofi, the contract promises associated with an option target would use a separate proportional performance model for purposes of revenue recognition under ASC 606. There was no significant financing component or non-cash consideration included in the Sanofi Agreement.

Under ASC 606, at the end of each reporting period, the Company re-evaluates the probability that the consideration associated with each milestone or reimbursement will not be subject to a significant reversal in the cumulative amount of revenue recognized, and, if necessary, adjusts the estimate of the overall transaction price. During the three months ended March 31, 2019, the Company reduced the overall transaction price by \$10.0 million. The transaction price includes the upfront, non-refundable payment of \$45.0 million for the transfer of the combined license, supply and development obligations under the Sanofi Agreement, an estimated \$32.6 million in reimbursable employee costs, an estimated \$54.5 million in reimbursable development costs including out-of-pocket costs paid to third parties and manufacturing costs and an estimated \$19.0 million in milestone payments.

Under ASC 606, the Company recognized revenue using the cost-to-cost input method, which it believes best depicts the transfer of control to the customer. Under the cost-to-cost input method, the extent of progress towards completion is measured based on the ratio of actual costs incurred to the total estimated costs expected upon satisfying the identified performance obligation. Under this method, revenue is recorded as a percentage of the estimated transaction price based on the extent of progress towards completion. The estimate of the Company's measure of progress and estimate of variable consideration to be included in the transaction price will be updated at each reporting date as a change in estimate. The amount related to the unsatisfied portion will be recognized as that portion is satisfied over time.

## Table of Contents

The following table summarizes the Company's collaboration revenue (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Collaboration revenue	\$ 1,174	\$ —	\$ 2,648	\$ —

The following table presents the balance of the Company's contract liabilities (in thousands):

	June 30, 2019	December 31, 2018
Contract liabilities		
Deferred revenue	\$43,207	\$ 44,413

The Company considers the total consideration expected to be earned in the next 12 months for services to be performed as short-term deferred revenue, and consideration that is expected to be earned subsequent to 12 months from the balance sheet date as long-term deferred revenue. The Company expects to complete its obligations and recognize all net revenues from the collaboration over eight years. Revenue recognized from contract liabilities was \$1.2 million during the six months ended June 30, 2019.

Included in prepaid expenses and other current assets on the condensed consolidated balance sheets as of June 30, 2019 and December 31, 2018 were \$0.7 million and \$0.8 million, respectively, of short-term receivables from Sanofi for reimbursable development costs.

## 4. Intangible Assets and Goodwill

### Intangible Assets, Net

The acquisition of Shire's MRT Program was accounted for in accordance with the acquisition method of accounting for business combinations. The total purchase consideration transferred was allocated to the tangible and identifiable intangible assets acquired based on their estimated fair values. The tables below present the Company's definite-lived intangible assets that are subject to amortization and indefinite-lived intangible assets:

	Estimated Life	June 30, 2019		
		Gross Carrying Amount	Accumulated Amortization	Net Carrying Amount
(In thousands)				
Definite-lived intangible assets:				
MRT	8 years	\$ 45,992	\$ (1,212)	\$ 44,780
Indefinite-lived intangible assets:				
IPR&D—CF	Indefinite	42,291	—	42,291
IPR&D—OTC	Indefinite	18,559	—	18,559
Total intangible assets, net		\$ 106,842	\$ (1,212)	\$ 105,630
(In thousands)				
	Estimated Life	December 31, 2018		
		Gross Carrying Amount	Accumulated Amortization	Net Carrying Amount
(In thousands)				
Definite-lived intangible assets:				
MRT	8 years	\$ 45,992	\$ (397)	\$ 45,595
Indefinite-lived intangible assets:				
IPR&D—CF	Indefinite	42,291	—	42,291
IPR&D—OTC	Indefinite	18,559	—	18,559
Total intangible assets, net		\$ 106,842	\$ (397)	\$ 106,445

Identifiable intangible assets acquired in the acquisition of Shire's MRT Program consisted of in-process research and development ("IPR&D"), which included ongoing projects that could further the Company's preclinical and clinical development activities related to CF, OTC deficiency and other potential rare diseases. As of the date of acquisition, the IPR&D was determined to

## Table of Contents

be indefinite-lived. Upon commencement of the Sanofi Agreement, the IPR&D—MRT intangible asset was reclassified from indefinite-lived to definite-lived intangible assets and the Company began amortization of this intangible asset. Amortization will be recorded over an estimated eight-year period based on an economic consumption model. For the three and six months ended June 30, 2019, the Company recorded amortization expense of \$0.3 million and \$0.8 million, respectively, related to the definite-lived MRT intangible asset. The estimated aggregate amortization expense for each of the five succeeding fiscal years is \$3.6 million, \$9.0 million, \$9.9 million, \$4.2 million, and \$2.3 million for the years ending December 31, 2019, 2020, 2021, 2022 and 2023, respectively.

Indefinite-lived IPR&D is not subject to amortization, but is tested annually for impairment or more frequently if there are indicators of impairment. The Company tests its indefinite-lived IPR&D annually for impairment on October 1st. In December 2018, the Company submitted an IND to the FDA to support the initiation of a Phase 1/2 clinical trial for MRT5201 in patients with OTC deficiency. In January 2019, the FDA notified the Company that its IND for MRT5201 was placed on clinical hold. The Company determined this clinical hold was an indicator of impairment and as a result, retested the indefinite-lived IPR&D related to the OTC deficiency program for impairment. The Company performed a quantitative impairment analysis whereby the Company forecasted the net cash flows expected to be generated by the indefinite-lived IPR&D related to the OTC deficiency program over its estimated useful life. The net cash flows reflected the program's stage of completion, the probability of technical success, the projected costs to complete, the expected market competition and an assessment of the program's life-cycle. The net cash flows were then adjusted to present value by applying an appropriate discount rate that reflects the risk factors associated with the cash flow streams. Following this retest the Company determined the indefinite-lived IPR&D related to the OTC deficiency program was not impaired. Therefore, the Company did not recognize an impairment charge. There were no indicators of impairment during the three months ended June 30, 2019.

### Goodwill

The excess of the fair value of the consideration transferred over the fair value of identifiable assets acquired in the acquisition of Shire's MRT Program was allocated to goodwill in the amount of \$21.4 million. There have been no changes to the carrying amount of goodwill during the six months ended June 30, 2019. Goodwill is not subject to amortization, but is tested annually for impairment or more frequently if there are indicators of impairment. The Company tests its goodwill annually for impairment on October 1st and concluded that goodwill was not impaired on October 1, 2018.

## 5. Fair Value of Financial Assets and Liabilities

The following tables present information about the Company's financial assets and liabilities measured at fair value on a recurring basis (in thousands):

	Fair Value Measurements as of June 30, 2019 Using:			
	Level 1	Level 2	Level 3	Total
<b>Assets:</b>				
Money market funds	\$ —	\$ 17,554	\$ —	\$ 17,554
U.S. government agency bonds	—	71,961	—	71,961
	<u>\$ —</u>	<u>\$ 89,515</u>	<u>\$ —</u>	<u>\$ 89,515</u>
<b>Liabilities:</b>				
Contingent consideration	\$ —	\$ —	\$120,233	\$120,233
	<u>\$ —</u>	<u>\$ —</u>	<u>\$120,233</u>	<u>\$120,233</u>
	Fair Value Measurements as of December 31, 2018 Using:			
	Level 1	Level 2	Level 3	Total
<b>Assets:</b>				
Money market funds	\$ —	\$ 23,318	\$ —	\$ 23,318
U.S. government agency bonds	—	88,904	—	88,904
	<u>\$ —</u>	<u>\$112,222</u>	<u>\$ —</u>	<u>\$112,222</u>
<b>Liabilities:</b>				
Contingent consideration	\$ —	\$ —	\$103,642	\$103,642
	<u>\$ —</u>	<u>\$ —</u>	<u>\$103,642</u>	<u>\$103,642</u>

## [Table of Contents](#)

During the six months ended June 30, 2019 and the year ended December 31, 2018, there were no transfers between Level 1, Level 2 and Level 3.

Cash equivalents as of June 30, 2019 and December 31, 2018 consisted of money market funds totaling \$17.6 million and \$23.3 million, respectively. The money market funds were valued using inputs observable in active markets for similar securities, which represent a Level 2 measurement in the fair value hierarchy. The Company's short-term investments as of June 30, 2019 and December 31, 2018 consisted of U.S. government agency bonds and were classified as available-for-sale securities. The U.S. government agency bonds were valued using inputs observable in active markets for similar securities, which represent a Level 2 measurement in the fair value hierarchy. As of June 30, 2019, the Company's short-term investments had an amortized cost of \$71.4 million, an unrealized gain of \$0.6 million and a fair value of \$72.0 million. All of these securities have a maturity of one year or less.

### ***Valuation of Contingent Consideration***

The contingent consideration liability related to the acquisition of Shire's MRT Program in 2016 was classified as Level 3 measurement within the fair value hierarchy. The Company may be required to pay future consideration contingent upon the achievement of potential future milestones and earnout payments that may be due by the Company to Shire.

The fair value of the liability to make potential future milestone and earnout payments was estimated by the Company at each reporting date based, in part, on the results of a third-party valuation using a discounted cash flow analysis based on various assumptions, including the probability of achieving specified events, discount rates, and the period of time until earnout payments are payable and the conditions triggering the milestone payments are met. The actual settlement of contingent consideration could differ from current estimates based on the actual occurrence of these specified events.

The following table presents the unobservable inputs and fair value of the components of the contingent consideration (dollar amounts in thousands):

	Unobservable Inputs at June 30, 2019 and December 31, 2018	Fair Value at	
		June 30, 2019	December 31, 2018
	Projected Year of Payment		
Earnout payments	2025 - 2039	\$110,770	\$ 94,999
Milestone payments	2025 - 2030	9,463	8,643
		<u>\$120,233</u>	<u>\$ 103,642</u>

The discount rate used in the third-party valuation was 13.5% and 14.5% as of June 30, 2019 and December 31, 2018, respectively.

The following table presents a roll-forward of the total acquisition-related contingent consideration liability (in thousands):

	Fair Value
Balance as of December 31, 2018	\$103,642
Change in fair value of contingent consideration	16,591
Balance as of June 30, 2019	<u>\$120,233</u>

The increase in the fair value of contingent consideration during the six months ended June 30, 2019 was primarily due to the continued progress of MRT5005 and MRT5201, the time value of money due to the passage of time and a decrease in the discount rate.

---

[Table of Contents](#)

## 6. Property and Equipment, Net

Property and equipment, net consisted of the following (in thousands):

	June 30, 2019	December 31, 2018
Laboratory equipment	\$ 8,394	\$ 7,012
Computer equipment	780	686
Office equipment	883	836
Leasehold improvements	5,634	5,635
Construction in progress	1,241	959
	16,932	15,128
Less: Accumulated depreciation and amortization	(6,029)	(4,883)
	<u>\$10,903</u>	<u>\$ 10,245</u>

Depreciation and amortization expense related to property and equipment was \$0.6 million, \$0.9 million, \$1.1 million and \$1.4 million for the three months ended June 30, 2019 and 2018 and the six months ended June 30, 2019 and 2018, respectively.

## 7. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	June 30, 2019	December 31, 2018
Accrued external research and development expenses	\$ 2,950	\$ 1,901
Accrued employee compensation and benefits	2,392	2,933
Accrued consultant and professional fees	1,904	977
Other	—	736
	<u>\$ 7,246</u>	<u>\$ 6,547</u>

## 8. Redeemable Convertible Preferred Stock

As of December 31, 2017, the Company had 142,288,292 shares of redeemable convertible preferred stock issued and outstanding which were redeemable and convertible by the holders under specified conditions. The redeemable convertible preferred stock was classified outside of stockholders' equity (deficit) because the shares contained redemption features that were not solely within the control of the Company.

Upon the closing of the Company's IPO on July 2, 2018, all then-outstanding shares of redeemable convertible preferred stock converted into an aggregate of 25,612,109 shares of common stock according to their terms. As of June 30, 2019 and December 31, 2018, there were no shares of redeemable convertible preferred stock authorized, issued or outstanding.

## 9. Incentive Stock Options and Restricted Stock

### 2018 Equity Incentive Plan

On March 7, 2018, the Company's board of directors, subject to stockholder approval, adopted, and on June 15, 2018, its stockholders approved, the 2018 Equity Incentive Plan (the "2018 Plan"), which became effective on June 27, 2018. The 2018 Plan provides for the grant of incentive stock options, non-qualified stock options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards.

The number of shares initially reserved for issuance under the 2018 Plan is the sum of 2,512,187, plus the number of shares (up to 1,013,167 shares) equal to the sum of (i) the number of shares remaining available for issuance under the 2016 Stock Incentive Plan, as amended, (the "2016 Plan"), upon the effectiveness of the 2018 Plan, which was 360,514 shares, and (ii) the number of shares of common stock subject to outstanding awards under the 2016 Plan that expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right. The number of shares of common stock that may be issued under the 2018 Plan will automatically increase on the first day of each fiscal year, beginning with the fiscal year ending December 31, 2019 and continuing for each fiscal year until, and including, the fiscal year

---

## [Table of Contents](#)

ending December 31, 2028, by an amount equal to the lowest of (i) 3,349,582 shares, (ii) 4% of the outstanding shares of common stock on such date and (iii) an amount determined by the Company's board of directors. Accordingly, on January 1, 2019, the number of shares of common stock that may be issued under the 2018 Plan increased by 1,805,598 shares of common stock and through June 30, 2019, a total of 87,364 shares issued under the 2016 plan have been cancelled for a total of 4,765,663 shares of common stock reserved for issuance under this plan as of June 30, 2019. The shares of common stock underlying any awards that are forfeited, canceled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, repurchased or are otherwise terminated by the Company under the 2018 Plan will be added back to the shares of common stock available for issuance under the 2018 Plan.

The 2018 Plan is administered by the board of directors. The exercise prices, vesting periods and other restrictions are determined at the discretion of the board of directors, except that the exercise price per share of options may not be less than 100% of the fair market value of the common stock on the date of grant. Stock options awarded under the 2018 Plan expire 10 years after the grant date, unless the board of directors sets a shorter term. Awards granted to employees, officers, members of the board of directors and consultants typically vest over a period of one to four years.

Typically, unvested stock options are forfeited upon the recipient ceasing to provide services to the Company.

### ***2018 Employee Stock Purchase Plan***

On March 7, 2018, the Company's board of directors, subject to stockholder approval, adopted, and on June 15, 2018, its stockholders approved the 2018 Employee Stock Purchase Plan (the "2018 ESPP"), which became effective on June 27, 2018. A total of 418,697 shares of common stock were initially reserved for issuance under this plan. The number of shares of common stock that may be issued under the 2018 ESPP will automatically increase on the first day of each fiscal year, beginning with the fiscal year commencing on January 1, 2019 and continuing for each fiscal year until, and including, the fiscal year commencing on January 1, 2029, by an amount equal to the lowest of (i) 837,395 shares, (ii) 1% of the outstanding shares of common stock on such date and (iii) an amount determined by the Company's board of directors. Accordingly, on January 1, 2019, the number of shares of common stock that may be issued under the 2018 ESPP increased by 451,399 shares for a total of 870,096 shares of common stock reserved for issuance under this plan.

As of June 30, 2019, no shares had been issued under the 2018 ESPP.

### ***2016 Stock Incentive Plan***

The 2016 Plan provides for the grant of stock options, stock appreciation rights, restricted stock and restricted stock units. Shares that are expired, terminated, surrendered or canceled under the 2016 Plan without having been exercised will be available for future grants of awards under the 2018 Plan. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for the grant of awards under the 2018 Plan.

The 2016 Plan is administered by the board of directors. The exercise prices, vesting periods and other restrictions were determined at the discretion of the board of directors, except that the exercise price per share of options could not be less than 100% of the fair market value of the common stock on the date of grant. Stock options awarded under the 2016 Plan expire 10 years after the grant date, unless the board of directors set a shorter term. Stock options and restricted stock granted to employees, officers, members of the board of directors and consultants typically vest over a four-year period.

Upon the effectiveness of the 2018 Plan on June 27, 2018, no further awards will be made under the 2016 Plan, but awards outstanding under the 2016 Plan will continue to be governed by their existing terms.

## Table of Contents

### Stock Options

The following table summarizes the Company's stock option activity since December 31, 2018 (in thousands, except share and per share amounts):

	<u>Number of Shares</u>	<u>Weighted Average Exercise Price</u>	<u>Weighted Average Remaining Contractual Term (in years)</u>	<u>Intrinsic Value</u>
Outstanding as of December 31, 2018	6,236,006	\$ 7.78	8.74	\$ 1,104
Granted	2,614,650	\$ 8.48		
Exercised	(221,401)	\$ 6.40		
Forfeited	(80,595)	\$ 7.85		
Outstanding as of June 30, 2019	<u>8,548,660</u>	\$ 8.03	8.83	\$39,344
Exercisable as of June 30, 2019	2,671,285	\$ 7.59	8.43	\$13,472
Vested and expected to vest as of June 30, 2019	8,548,660	\$ 8.03	8.83	\$39,344
Exercisable as of December 31, 2018	1,827,004	\$ 7.16	7.95	\$ 632
Vested and expected to vest as of December 31, 2018	6,236,006	\$ 7.78	8.74	\$ 1,104

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the options and the fair value of the Company's common stock for those options that had exercise prices lower than the fair value of the Company's common stock. The aggregate intrinsic value of stock options exercised during the six months ended June 30, 2019 and 2018 was \$0.6 million and \$0.3 million, respectively.

The weighted average grant-date fair value per share of stock options granted was \$5.56 and \$5.91 during the six months ended June 30, 2019 and 2018, respectively.

### Stock Option Valuation

The fair value of stock option grants is estimated using the Black-Scholes option-pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The expected term of stock options granted to non-employees is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

The following table presents, on a weighted average basis, the assumptions used in the Black-Scholes option-pricing model to determine the grant-date fair value of stock options granted to employees and directors:

	<u>Six Months Ended June 30,</u>	
	<u>2019</u>	<u>2018</u>
Risk-free interest rate	2.42%	2.80%
Expected term (in years)	6.0	6.0
Expected volatility	73.3%	75.7%
Expected dividend yield	0%	0%

---

[Table of Contents](#)**Restricted Common Stock**

The following table summarizes the Company's restricted stock activity since December 31, 2018:

	Number of Shares	Weighted Average Grant-Date Fair Value
Unvested restricted common stock outstanding as of December 31, 2018	219,148	\$ 1.27
Forfeited restricted common stock	(1,334)	\$ 1.28
Vested restricted common stock	(97,826)	\$ 1.27
Unvested restricted common stock outstanding as of June 30, 2019	<u>119,988</u>	\$ 1.28

**Stock-Based Compensation**

Stock-based compensation expense was classified in the condensed consolidated statements of operations as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Research and development expenses	\$ 1,284	\$ 1,671	\$ 2,153	\$ 2,453
General and administrative expenses	2,265	1,204	3,356	1,805
	<u>\$ 3,549</u>	<u>\$ 2,875</u>	<u>\$ 5,509</u>	<u>\$ 4,258</u>

Included in general and administrative stock-based compensation expense during each of the three and six months ended June 30, 2019 is \$0.8 million related to the issuance of 67,406 shares of common stock in connection with a former employee letter agreement.

As of June 30, 2019, total unrecognized compensation cost related to the unvested stock-based awards was \$29.3 million, which is expected to be recognized over a weighted average period of 2.75 years.

**10. Income Taxes**

The Company recognized an income tax benefit of \$1.5 million during the three months ended June 30, 2018 and \$0.5 million and \$2.6 million during the six months ended June 30, 2019 and 2018, respectively. There was no income tax benefit recognized during the three months ended June 30, 2019. The income tax benefits recognized during the three months ended June 30, 2018 and six months ended June 30, 2019 and 2018 resulted from a reduction in the deferred tax liabilities recorded as part of the Company's acquisition of the MRT Program as well as deferred tax assets recorded for net operating losses generated that have an unlimited carryforward period. Net operating losses generated in 2018 and years thereafter can be carried forward indefinitely. The reduction in the deferred tax liabilities during the three months ended June 30, 2018 and six months ended June 30, 2019 and 2018 resulted from an increase in the tax basis of the indefinite-lived IPR&D recorded in the acquisition.

[Table of Contents](#)

**11. Net Loss per Share**

*Net Loss per Share*

Basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share amounts):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
<b>Numerator:</b>				
Net loss	\$ (27,832)	\$ (27,503)	\$ (61,030)	\$ (48,712)
Accretion of redeemable convertible preferred stock to redemption value	—	(459)	—	(644)
Net loss attributable to common stockholders	<u>\$ (27,832)</u>	<u>\$ (27,962)</u>	<u>\$ (61,030)</u>	<u>\$ (49,356)</u>
<b>Denominator:</b>				
Weighted average common shares outstanding—basic and diluted	<u>48,749,627</u>	<u>9,187,207</u>	<u>46,866,842</u>	<u>9,139,638</u>
Net loss per share attributable to common stockholders—basic and diluted	<u>\$ (0.57)</u>	<u>\$ (3.04)</u>	<u>\$ (1.30)</u>	<u>\$ (5.40)</u>

Due to a clerical error in the computation of the net loss attributable to common stockholders, the Company reported the net loss per share applicable to common stockholders—basic and diluted for the three and six months ended June 30, 2018 as \$2.94 and \$5.26, respectively, in the Company’s Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2018. The net loss per share applicable to common stockholders—basic and diluted for the three and six months ended June 30, 2018 was \$3.04 and \$5.40, respectively, as shown in the chart above. The Company concluded that this error was not material to the periods impacted.

The Company excluded 147,914 shares and 412,040 shares of restricted common stock, presented on a weighted average basis, from the calculations of basic net loss per share attributable to common stockholders for the three months ended June 30, 2019 and 2018, respectively, because those shares had not vested. The Company excluded 172,941 shares and 451,286 shares of restricted common stock, presented on a weighted average basis, from the calculations of basic net loss per share attributable to common stockholders for the six months ended June 30, 2019 and 2018, respectively, because those shares had not vested.

The Company’s potentially dilutive securities, which include stock options, unvested restricted common stock and redeemable convertible preferred stock, have been excluded from the computation of diluted net loss per share attributable to common stockholders as the effect would be to reduce the net loss per share. Therefore, the weighted average number of shares of common stock outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same.

The Company excluded the following potential shares of common stock, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	June 30,	
	2019	2018
Options to purchase common stock	8,548,660	6,093,408
Unvested restricted common stock	119,988	368,875
Redeemable convertible preferred stock (as converted to common stock)	—	25,612,109
	<u>8,668,648</u>	<u>32,074,392</u>

In addition to the potentially dilutive securities noted above, as of June 30, 2018, the Company was obligated to issue common stock to Shire upon the occurrence of specified events. Because the necessary conditions for issuance of the shares had not been met as of June 30, 2018, the Company excluded these shares from the table above and from the calculations of diluted net loss per share for the three and six months ended June 30, 2018. The Company’s obligation to issue additional shares of its common stock to Shire was satisfied upon completion of the Company’s IPO.

## 12. Leases

The Company is a lessee under two operating leases comprising a commercial real estate lease and an equipment lease.

### *Real Estate Lease*

In June 2017, the Company entered into an operating lease for office and laboratory space at its headquarters in Lexington, Massachusetts. The Company occupies approximately 59,000 square feet of space under a 10-year lease agreement expiring in April 2028. The Company occupied this property in March 2018. Monthly lease payments include base rent charges of \$0.2 million, which are subject to a 3% annual increase each year. In June 2017, in connection with this lease agreement, the Company issued a letter of credit collateralized by cash deposits of \$1.0 million, which are classified as restricted cash on the consolidated balance sheets as of June 30, 2019 and December 31, 2018.

### *Equipment Lease*

In March 2018, the Company entered into an operating lease for communications equipment for use at its office and laboratory space in Lexington, Massachusetts. The term of the lease is five years, expiring in March 2023.

The Company excludes leases with an initial term of one year or less in the recognized ROU assets and lease liabilities. The Company recognizes lease expense for these leases on a straight-line basis over the lease term. For lease agreements entered into or reassessed after the adoption of ASC 842, lease and non-lease components are combined into a single lease component. The Company's leases have remaining lease terms of up to nine years, excluding two five-year options to extend the real estate lease after the expiration of the initial term. The Company believes this office and laboratory space will be sufficient to meet its needs for the foreseeable future and that suitable additional space will be available as and when needed.

The components of lease cost were as follows (dollar amounts in thousands):

	Three Months Ended June 30, 2019	Six Months Ended June 30, 2019
<b>Lease Cost</b>		
Operating lease cost	\$ 673	\$ 1,346
Short-term lease cost	—	—
<b>Total lease cost</b>	<u>\$ 673</u>	<u>\$ 1,346</u>
<b>Other Information</b>		
Operating cash flows from operating leases	\$ 650	\$ 1,282
Operating lease liabilities arising from obtaining right-of-use assets	\$ —	\$ —
Weighted-average remaining lease term	9 years	9 years
Weighted-average discount rate	17.5%	17.5%

During the three and six months ended June 30, 2018, the company recorded rent expense of \$0.8 million and \$1.7 million, respectively.

As of June 30, 2019, minimum rental commitments under these leases are as follows (in thousands):

	June 30, 2019
2019	\$ 1,301
2020	2,659
2021	2,737
2022	2,818
2023	2,860
2024 and thereafter	<u>13,096</u>
<b>Total future minimum lease payments</b>	<b>25,471</b>
Less: imputed interest	<u>(12,652)</u>
<b>Present value of lease liabilities</b>	<b>\$ 12,819</b>

---

## [Table of Contents](#)

As of December 31, 2018, minimum rental commitments under the real estate lease was as follows (in thousands):

	<b>December 31, 2018</b>
2019	\$ 2,534
2020	2,610
2021	2,688
2022	2,769
2023	2,852
2024 and thereafter	13,096
<b>Total future minimum lease payments</b>	<b>\$ 26,549</b>

As most of the Company's leases do not provide an implicit rate, the Company uses its incremental borrowing rate which are the rates incurred to borrow on a collateralized basis over a term equal to the lease payments in a similar economic environment in determining the present value of lease payments. The Company used the incremental borrowing rate on January 1, 2019 for operating leases that commenced prior to that date.

### **13. Commitments and Contingencies**

#### *Research, Supply and License Agreements*

Pursuant to the Shire Agreement in December 2016, the Company was assigned and assumed several contracts related to the MRT Program. The material agreements that were assigned to and assumed by the Company in connection with the acquisition are described below.

#### *Roche Master Supply Agreement*

The Company is a party to a master supply agreement with Roche Diagnostics Corporation ("Roche") pursuant to which Roche will custom manufacture certain products for the Company. The agreement requires the Company to purchase from Roche specified manufactured products and the related raw materials in an amount equal to the greater of (i) quantities of raw materials in the Company's annual forecast to be purchased or (ii) 80% of the Company's demand for products as the same or similar type (the "Purchase Commitment"). In June 2017, the Company exercised its option under the agreement to extend the agreement through December 31, 2024. In September 2018, the Company and Roche amended the agreement to remove and replace the Purchase Commitment for certain manufactured products and related raw materials supplied by Roche. The agreement, as amended, specifies a minimum purchase requirement for certain custom manufactured products. As of June 30, 2019, the Company's purchase commitments under the agreement totaled \$20.4 million, with \$6.0 million committed as payments for the remainder of 2019, \$0.5 million committed as payments in 2020 and \$3.5 million committed as payments each year from 2021 to 2024. Research and development expenses related to this agreement totaled \$1.0 million and \$0.8 million during the three months ended June 30, 2019 and 2018, respectively, and \$3.5 million and \$1.7 million during the six months ended June 30, 2019 and 2018, respectively.

#### *MIT Research Agreement*

The Company is a party to a research agreement with the Massachusetts Institute of Technology ("MIT") pursuant to which the Company is obligated to reimburse MIT in an amount up to \$3.1 million for specified direct and indirect costs incurred through October 2019 in specified research activities conducted for the Company. As of June 30, 2019 and December 31, 2018, the Company had paid MIT \$3.1 million and \$2.5 million, respectively, of the total committed amount. Research and development expenses related to this agreement totaled \$0.2 million and \$0.3 million during the three months ended June 30, 2019 and 2018, respectively, and \$0.5 million and \$0.6 million during the six months ended June 30, 2019 and 2018, respectively. As of June 30, 2019 and December 31, 2018, there were no amounts payable by the Company under the agreement.

As amended, the agreement expires in October 2019 and may be extended thereafter by mutual agreement of the parties.

---

## [Table of Contents](#)

### *MIT Exclusive Patent License Agreement*

The Company is a party to an exclusive patent license agreement with MIT pursuant to which the Company received an exclusive license under the licensed patent rights to develop, manufacture and commercialize any product containing both (i) any RNA sequences, including mRNA, that encode a protein or peptide suitable for human therapeutic use which may include operably linked non-coding sequences that facilitate translation of the coding portion of such RNA sequence, but such non-coding sequences do not include nucleic acids that function through an RNA interface mechanism or transcriptional activation mechanism (the “coding RNA component”), and (ii) products covered by the licensed patent rights (the “lipid products”). A product containing both a coding RNA component and a lipid product is referred to as a “licensed product.” Under the licensed patent rights, the Company is permitted to develop, manufacture and commercialize the licensed products for the delivery of coding RNA components to treat disease in humans.

The Company has the right to grant sublicenses under this license. The patent rights licensed to the Company by MIT include claims that cover the Company’s customized lipid-based nanoparticles used for delivery of coding RNA components in its MRT platform and MRT5201.

Under the license agreement, the Company is obligated to make annual license maintenance payments to MIT, payable on January 1 of each calendar year, of up to \$0.2 million, which may be credited against royalties subsequently due on net sales of licensed products earned in the same calendar year. The Company paid no annual license maintenance fees to MIT during each of the three months ended June 30, 2019 and 2018 and paid \$0.2 million and \$0.1 million during the six months ended June 30, 2019 and 2018, respectively.

The Company is also obligated to make milestone payments to MIT aggregating up to \$1.375 million upon the achievement of specified clinical and regulatory milestones with respect to each licensed product and \$1.250 million upon the Company’s first commercial sale of each licensed product, and to pay royalties of a low single-digit percentage to MIT based on the Company’s, and any of its affiliates’ and sublicensees’, net sales of licensed products. The royalties are payable on a product-by-product and country-by-country basis, and may be reduced in specified circumstances. The Company’s obligation to make royalty payments extends with respect to a licensed product in a country until the expiration of the last-to-expire patent or patent application licensed from MIT covering the licensed product in the country. In addition, the Company is obligated to pay MIT a low double-digit percentage of the portion of income from sublicensees that the Company ascribes to the MIT-licensed patents, excluding royalties on net sales and research support payments. Pursuant to such provision, the Company agreed to pay \$0.7 million to MIT as MIT’s share of sublicense income with respect to the upfront payment received under the Sanofi Agreement. The amounts that the Company may owe to MIT will depend upon the relative value of the patents the Company licensed from MIT and sublicensed to Sanofi as compared to the other rights that the Company licensed to Sanofi. The determination of the relative value of such rights is subject to a process described in the Company’s license agreement with MIT (see Note 3).

The agreement obligates the Company to use commercially reasonable efforts and expend a minimum amount of resources each year to develop licensed products in accordance with a development plan, and a development milestone timetable specified in the agreement; to use commercially reasonable efforts to commercialize licensed products; and upon commercialization, to make the licensed products reasonably available to the public.

MIT has the right to terminate the agreement if the Company fails to pay amounts when due or otherwise materially breaches the agreement and fails to cure such nonpayment or breach within specified cure periods or in the event the Company ceases to carry on its business related to the agreement. In the event of a termination due to the Company’s breach caused by a due diligence failure of a licensed product, but where the Company has fulfilled its obligations with respect to a different licensed product, MIT may not terminate the agreement with respect to the different licensed product. MIT may immediately terminate the agreement if the Company or any of its affiliates brings specified patent challenges against MIT or assists others in bringing a patent challenge against MIT. The Company has the right to terminate the agreement for its convenience at any time on three months’ prior written notice to MIT and payment of all amounts due to MIT through the date of termination.

The Company’s patent rights, and the rights of its affiliates and sublicensees, in specified licensed products may also terminate, if the Company, its affiliates or MIT receives a request from a third party to develop such licensed product for which the Company is unable to, within nine months of receiving notice of any such request, either demonstrate that the Company has initiated a fully funded project for the commercial development of such licensed product, and provide a business plan with acceptable milestones; demonstrate that the licensed product proposed by such third party would be competitive with a licensed product for which the Company has initiated a fully funded project; or enter into a sublicense agreement with such third party on commercially reasonable terms, and, in each case, MIT, in its sole discretion, grants a license to such third party for the specified patent rights.

---

## [Table of Contents](#)

Research and development expenses related to this agreement totaled less than \$0.1 million during each of the three months ended June 30, 2019 and 2018, and \$0.1 million during each of the the six months ended June 30, 2019 and 2018, respectively. As of June 30, 2019 and December 31, 2018, there were no liabilities recorded by the Company related to this agreement.

### ***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 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 does not believe that the outcome of any claims under indemnification arrangements will have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of June 30, 2019 and December 31, 2018.

### ***Legal Proceedings***

The Company is not a party to any litigation and does not have contingency reserves established for any litigation liabilities.

## **14. Related Party Transactions**

### ***Consulting Agreement with Daniel S. Lynch***

In 2012, the Company entered into a consulting agreement with Daniel S. Lynch, the chairman of the Company's board of directors, for the provision of consulting, advisory and related services. Pursuant to the consulting agreement, as amended through March 2015, Mr. Lynch was entitled to base compensation of \$100,000 per year and was eligible to receive an annual performance bonus of up to 25% of his base compensation. In June 2018, the Company's board of directors approved a director compensation program that became effective on the effective date of the registration statement related to the Company's IPO. The Company has not made any payments to Mr. Lynch under the consulting agreement since the approval of the director compensation program. During the three and six months ended June 30, 2019 and 2018, the Company recorded general and administrative expenses of \$0, \$31,250, \$0 and \$0.1 million, respectively, related to this agreement. During the three and six months ended June 30, 2019 and 2018, the Company paid Mr. Lynch \$0, \$0.1 million, \$11,250 and \$0.1 million, respectively, in connection with his services provided under the agreement. As of June 30, 2019 and December 31, 2018, amounts due under this agreement totaled \$0 and \$11,250, respectively, which were included in accrued expenses on the consolidated balance sheets.

### ***Private Placement***

In connection with a private placement of the Company's common stock, entities affiliated with Baupost Group, L.L.C., a substantial stockholder, purchased 2,352,941 shares of the Company's common stock at a price per share of \$8.50 for an aggregate purchase price of \$20.0 million.

## **15. Subsequent Event**

On July 3, 2019, the Company filed a universal shelf registration statement on Form S-3 with the SEC (the "2019 Shelf") to register for sale from time to time up to \$250.0 million of common stock, preferred stock, debt securities, warrants and/or units in one or more offerings, which became effective on July 19, 2019 (File No. 333-232543).

On July 3, 2019, the Company entered into an Open Market Sale Agreement<sup>SM</sup> (the "Sales Agreement") with Jefferies LLC ("Jefferies") under which the Company may issue and sell shares of common stock, from time to time, having an aggregate offering price of up to \$50.0 million. The Company has not sold any shares under the Sales Agreement as of July 29, 2019. Sales of common stock through Jefferies may be made by any method that is deemed an "at the market" offering as defined in Rule 415 promulgated under the Securities Act of 1933, as amended. Jefferies has agreed to use its commercially reasonable efforts consistent with its normal trading and sales practices to sell the common stock based upon the Company's instructions. The Company is not obligated to make any sales of its common stock under the Sales Agreement. Any shares sold would be pursuant to the 2019 Shelf.

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

*You should read the following discussion and analysis of our financial condition and results of operations together with our unaudited condensed financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and our audited financial statements and related notes included in our Annual Report on Form 10-K for the year ended December 31, 2018, or the 2018 Annual Report, that was filed with the Securities and Exchange Commission, or SEC, on March 21, 2019.*

*Some of the statements contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business, constitute forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. We have based these forward-looking statements on our current expectations and projections about future events. The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this Quarterly Report on Form 10-Q, particularly including those risks identified in Part II-Item 1A "Risk Factors" and our other filings with the SEC.*

*Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report on Form 10-Q. Statements made herein are as of the date of the filing of this Form 10-Q with the SEC and should not be relied upon as of any subsequent date. Even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report on Form 10-Q, they may not be predictive of results or developments in future periods. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.*

### **Overview**

We are a clinical-stage messenger RNA, or mRNA, therapeutics company developing a new class of potentially transformative medicines to treat diseases caused by protein or gene dysfunction. Using our proprietary mRNA therapeutic platform, or MRT platform, we create mRNA that encodes functional proteins. We believe that the mRNA design, delivery and manufacturing capabilities of our MRT platform provide us with the most advanced platform for developing product candidates that deliver mRNA encoding functional proteins for therapeutic uses. Our mRNA is delivered to the target cell where the cell's own machinery recognizes it and translates it, restoring or augmenting protein function to treat or prevent disease. We believe that our MRT platform is broadly applicable across multiple diseases in which the production of a desirable protein can have a therapeutic effect. We are initially focused on restoring the expression of intracellular and transmembrane proteins, areas that have eluded conventional protein therapeutics, in patients with genetic diseases where there is high unmet medical need.

We are developing our lead MRT product candidate for the lung, MRT5005, for the treatment of cystic fibrosis, or CF. We are conducting a Phase 1/2 clinical trial to evaluate the safety and tolerability of MRT5005. Percent predicted forced expiratory volume in one second, or ppFEV<sub>1</sub>, which is a well-defined and accepted endpoint measuring lung function, is also being measured at pre-defined timepoints throughout the trial. In April 2019, we completed dosing of patients in the single-ascending dose, or SAD, portion of the Phase 1/2 clinical trial and on July 31, 2019, we reported interim data from the SAD portion of the clinical trial. MRT5005 was generally well tolerated at low and mid-dose levels with no serious adverse events reported at any dose level. Marked increases in ppFEV<sub>1</sub> were observed after a single dose of MRT5005, primarily at mid-dose. Based on the analysis of the interim results, we plan to amend the clinical trial protocol to include two additional cohorts of patients. In early 2019, we began dosing patients in the multiple-ascending dose, or MAD, portion of this trial, and we expect to report results from the additional SAD cohort and the ongoing MAD portion of the clinical trial in 2020.

We are developing our lead MRT product candidate for the liver, MRT5201, for the treatment of ornithine transcarbamylase, or OTC, deficiency. In June 2019, we received clearance from the U.S. Food and Drug Administration, or FDA, to proceed with a SAD Phase 1/2 clinical trial of MRT5201 in adult patients with OTC deficiency. In December 2018, we had submitted an investigational new drug application, or IND, for a SAD and MAD Phase 1/2 clinical trial of MRT5201, which the FDA placed on clinical hold, pending additional preclinical toxicology data. After discussions with the FDA and after we amended the protocol, the FDA removed the clinical hold and allowed us to move forward with a SAD clinical trial. We plan to initiate patient screening for the SAD Phase 1/2 clinical trial in the second half of 2019. We are currently conducting additional preclinical studies that are required to support future clinical development of MRT5201, including a MAD clinical trial, and plan to submit data from these preclinical studies to the FDA in the fourth quarter of 2019.

Additionally, we intend to leverage the broad applicability of our platform by identifying lead preclinical candidates for additional lung and liver disease targets, and through a collaboration with Sanofi Pasteur Inc., or Sanofi, the vaccines global business unit of Sanofi S.A., to develop infectious disease vaccines using mRNA technology for up to five infectious disease pathogens. We have several discovery-stage programs to identify additional potential mRNA therapeutic candidates. We believe that our MRT platform is distinct from other mRNA-based technologies and has the potential to provide clinical benefits by transforming life-threatening illnesses into manageable chronic conditions.

---

## [Table of Contents](#)

Since our inception in 2011, we have devoted substantially all of our focus and financial resources to organizing and staffing our company, business planning, raising capital, acquiring or discovering product candidates and securing related intellectual property rights and conducting discovery, research and development activities for our programs. We do not have any products approved for sale and have not generated any revenue from product sales.

In 2018, we entered into a collaboration and license agreement with Sanofi, or the Sanofi Agreement, to develop mRNA vaccines for up to five infectious disease pathogens. Under the Sanofi Agreement, we and Sanofi are jointly conducting research and development activities to advance mRNA vaccines and mRNA vaccine platform development during a three-year research term, which may be extended by mutual agreement. We are eligible to receive up to \$805.0 million in payments, including an upfront payment of \$45.0 million, which we received in 2018; certain development, regulatory and sales-related milestones across several vaccine targets, and option exercise fees if Sanofi exercises its option related to development of vaccines for additional pathogens. We are also eligible to receive reimbursable development costs and tiered royalty payments associated with worldwide sales of the developed vaccines, if any.

Through June 30, 2019, we have funded our operations primarily with net cash proceeds of \$189.2 million from the sale of redeemable convertible preferred stock and the sale of bridge units, which ultimately converted into shares of our common stock, net cash proceeds of \$113.2 million from our initial public offering of our common stock, or the IPO, \$45.0 million from the upfront payment received under the Sanofi Agreement and net cash proceeds of \$44.1 million from a private placement of our common stock.

Since our inception, we have incurred significant operating losses. Our ability to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of our current or future product candidates. Our net losses were \$27.8 million and \$27.5 million for the three months ended June 30, 2019 and 2018, respectively, and \$61.0 million and \$48.7 million for the six months ended June 30, 2019 and 2018, respectively. As of June 30, 2019, we had an accumulated deficit of \$307.2 million. We expect to continue to incur significant expenses for at least the next several years as we advance our product candidates from discovery through preclinical development and clinical trials and seek regulatory approval of our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. We may also incur expenses in connection with the in-licensing or acquisition of additional product candidates.

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 the sale of equity, debt financings or other capital sources, including collaborations, strategic partnerships or marketing, distribution or licensing arrangements with third parties or grants from organizations and foundations. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates or delay our pursuit of potential in-licenses or acquisitions.

Because of the numerous risks and uncertainties associated with product development, we are unable to 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. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of June 30, 2019, we had cash, cash equivalents and short-term investments of \$146.9 million. We believe that our existing cash, cash equivalents and short-term investments will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2020. In accordance with the requirements of Accounting Standards Update, or ASU, No. 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40)*, or ASC 205-40, we have determined that there is substantial doubt about our ability to continue as a going concern. The financial statements do not include any adjustments that might result from the outcome of this uncertainty.

There is no assurance that we will be successful in obtaining additional financing on terms acceptable to us, if at all, nor is it considered probable under the accounting standards. As such, under the requirements of ASC 205-40, management may not consider the potential for future capital raises or management plans to reduce costs that are not considered probable in their assessment of our ability to meet our obligations. If we are unable to obtain funding, we may be required to delay, reduce or eliminate our product development or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves, which could adversely affect our business prospects, and we may be unable to continue operations. See “—Liquidity and Capital Resources—Funding Requirements.”

---

[Table of Contents](#)**Recent Developments*****Interim Results from Phase 1/2 Clinical Trial of MRT5005 in Patients with Cystic Fibrosis***

On July 31, 2019, we reported interim results from the SAD portion of our Phase 1/2 clinical trial of MRT5005 for the treatment of patients with CF. The interim results summarize data from 12 adult patients with CF who received a single dose of either MRT5005 or placebo (3:1 randomization). Patients who received MRT5005 were assigned to one of three dose groups (8, 16 or 24 mg). Of the 12 patients, 11 had at least one copy of the F508del mutation and one patient did not have a F508del mutation and was considered non-amenable to cystic fibrosis transmembrane conductance regulator, or CFTR, modulator treatment. Seven of the 12 patients were taking an approved CFTR modulator through screening, dosing and follow-up.

***Safety, Tolerability and Pharmacokinetic Summary***

The most common adverse events through Day 29 were cough and headache. There were no treatment-emergent serious adverse events, or SAEs. All treatment-emergent adverse events, or TEAEs were considered mild to moderate. Five patients, 3 of whom were in the 24 mg dose group, experienced transient, mild to moderate febrile reactions deemed related to study drug. These events occurred approximately 4-10 hours post dosing, and were characterized by fever and symptoms such as headache, fatigue, chills or nausea, which were treated with medicines such as acetaminophen and nonsteroidal anti-inflammatory drugs, or NSAIDs, or an anti-emetic. Symptoms resolved within 24 hours, and all patients were discharged from the study center on Day 2 as planned. In these five patients, low levels of mRNA and/or lipid were detected in the blood.

<b>Characteristics through Day 29</b>	<b>MRT5005 8 mg (N=3)</b>	<b>MRT5005 16 mg (N=3)</b>	<b>MRT5005 24 mg (N=3)</b>	<b>Pooled Placebo (N=3)</b>
Overall Number of TEAEs reported	28	25	33	11
Not related	11	9	10	9
Related	17	16	23	2
Number of Serious TEAEs reported	0	0	0	0
Number of TEAEs leading to discontinuation	0	0	0	0
Number of TEAEs resulting in death	0	0	0	0
Number of TEAEs classified as pulmonary exacerbation	0	0	2*	0
Number of TEAEs by Severity				
Mild	23	24	21	11
Moderate	5	1	12	0
Severe	0	0	0	0

\* Occurred at Day 25 and Day 27

## Table of Contents

### Lung Function (ppFEV<sub>1</sub>) Summary

A primary measure of lung function, ppFEV<sub>1</sub>, was assessed at pre-defined timepoints throughout the trial. Patients in the pooled placebo group and the 8 mg dose group did not show a marked improvement in ppFEV<sub>1</sub>. In the eight day period after dosing, the three patients in the 16 mg dose group demonstrated maximal ppFEV<sub>1</sub> increases of 11.1%, 13.6% and 22.2%, for a mean maximum increase from baseline (+/- standard deviation) of 15.7% (5.8). Of the three patients in the 16 mg dose group, two were on a stable CFTR modulator treatment regimen for at least 28 days, while the third had a genotype that is considered non-amenable to CFTR modulator treatments. Of the three patients in the 24 mg dose group, through Day 8, one patient experienced a maximum increase in ppFEV<sub>1</sub> from baseline of 21.4%, while two patients did not show a marked increase in ppFEV<sub>1</sub>.

	Mean Baseline ppFEV <sub>1</sub> % (SD)	Absolute Change from Baseline Mean ppFEV <sub>1</sub> % (SD)				Maximum Change from Baseline through Day 8 Mean ppFEV <sub>1</sub> % (SD)
		Day 1 *	Day 2	Day 3	Day 8	
<b>8 mg</b>					-	
	53.3 (7.2)	3.7 (0.9)	2.8 (1.8)	3.4 (2.3)	1.2 (3.0)	4.4 (0.7)
<b>16 mg</b>	72.0 (6.6)	7.2 (7.3)	11.2 (10.3)	11.4 (5.1)	9.2 (1.8)	15.7 (5.8)
<b>24 mg</b>	79.2 (7.1)	2.5 (0.4)	8.6 (11.2)	6.6 (4.8)	2.3 (3.2)	9.7 (10.2)
<b>Pooled Placebo</b>					-	
	60.5 (18.5)	2.0 (3.8)	0.4 (2.4)	-0.2 (1.4)	0.6 (4.0)	3.2 (2.7)

\* 8 hours post dose

### Phase 1/2 Clinical Trial Design

Based on the analysis of the SAD (Part A) interim results, we have proposed certain protocol changes in this ongoing Phase 1/2 clinical trial. In the SAD portion of the trial, we plan to amend the clinical trial protocol to include an additional 20 mg single dose cohort of four patients. In the MAD portion of the trial (Part B), we plan to amend the clinical trial protocol to add 12 and 20 mg dose cohorts with four patients each, the latter dosing cohort contingent on successful completion of the 20 mg single dose cohort and an acceptable safety profile in the lower MAD dose cohorts. We no longer plan to evaluate a 24 mg dose group in the MAD portion of the trial. The originally planned Part C of the clinical trial, which included bronchoscopies, is expected to be redesigned as a Part B expansion to instead focus on the enrollment of additional patients at dose levels of interest. As required, we plan to submit these protocol changes to the FDA. We expect to report results from the additional 20 mg SAD cohort and the ongoing MAD portion of the trial in 2020.

The randomized, double-blind, placebo-controlled Phase 1/2 clinical trial of MRT5005 was designed to enroll at least 32 adult patients with CF who have two Class I and/or Class II mutations. After accounting for the planned protocol amendments, we expect to enroll up to 40 adult patients with CF. The primary endpoint of the trial is to assess the safety and tolerability of single and multiple escalating doses of MRT5005 administered by nebulization. ppFEV<sub>1</sub>, which is a well-defined and accepted endpoint measuring lung function, is also being measured at pre-defined timepoints throughout the trial. The Phase 1/2 clinical trial of MRT5005 for the treatment of patients with CF is being conducted in collaboration with the Cystic Fibrosis Foundation Therapeutics Development Network.

## **Components of Our Results of Operations**

### ***Revenue from Product Sales***

To date, we have not generated any revenue from product sales, and we do not expect to generate any revenue from the sale of products in the near future. If our development efforts for our product candidates are successful and result in regulatory approval, we may generate revenue in the future from product sales.

### ***Collaboration Revenue***

In 2018, we entered into the Sanofi Agreement, a collaboration and license agreement with Sanofi to develop mRNA vaccines and mRNA vaccine platform development for up to five infectious disease pathogens, or the Licensed Fields.

Under the terms of the Sanofi Agreement, we have granted to Sanofi exclusive, worldwide licenses under applicable patents, patent applications, know-how and materials, including those arising under the collaboration, to develop, commercialize and manufacture mRNA vaccines to prevent, treat or cure diseases, disorders or conditions in humans caused by any of three Licensed Fields. In addition, pursuant to the terms of the Sanofi Agreement and subject to certain limitations, Sanofi has the options to add up to two additional infectious disease pathogens within the granted licenses to the License Fields.

Under revenue recognition guidance, we account for: (i) the license we conveyed to Sanofi with respect to the Licensed Fields, (ii) the licensed know-how to be conveyed to Sanofi with respect to the Licensed Fields, (iii) our obligations to perform research and development on the Licensed Fields, (iv) our obligation to transfer licensed materials to Sanofi, (v) our obligation to manufacture and supply certain non-clinical and clinical mRNA vaccines and materials containing mRNA until we transfer such manufacturing capabilities to Sanofi and (vi) the technology and process transfer as a single performance obligation. We recognize revenue using the cost-to-cost input method, which we believe best depicts the transfer of control to the customer. Under the cost-to-cost input method, the extent of progress towards completion is measured based on the ratio of actual costs incurred to the total estimated costs expected upon satisfying the identified performance obligation. Under this method, revenue is recorded as a percentage of the estimated transaction price based on the extent of progress towards completion.

### ***Operating Expenses***

#### ***Research and Development Expenses***

Research and development expenses consist primarily of costs incurred in connection with the discovery and development of our product candidates. We expense research and development costs as incurred. These expenses include:

- employee-related expenses, including salaries, related benefits 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 third parties, such as consultants and contract research organizations, or CROs;
- the cost of manufacturing drug products for use in our preclinical studies and clinical trials, including under agreements with third parties, such as consultants and contract manufacturing organizations, or CMOs;
- laboratory supplies;
- facilities, depreciation and other expenses, which include direct or allocated expenses for rent and maintenance of facilities and insurance;
- costs to fulfill our obligations under the Sanofi Agreement;
- costs related to compliance with regulatory requirements; and
- payments made under third-party licensing agreements.

## Table of Contents

We recognize external development costs based on an evaluation of the progress to completion of specific tasks using information provided to us by our service providers. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such amounts are recognized as an expense when the services have been performed or the goods have been delivered, or when it is no longer expected that the goods will be delivered or the services rendered. Upfront payments, milestone payments (other than those deemed contingent consideration in a business combination) and annual maintenance fees under license agreements are expensed in the period in which they are incurred.

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 outside consultants, CROs, CMOs and central laboratories in connection with our preclinical development, process development, manufacturing and clinical development activities. Our direct research and development expenses by program also include costs of laboratory supplies incurred for each program as well as fees incurred under license agreements. We do not allocate employee costs or facility expenses, including depreciation or other indirect costs, to specific programs because these costs are deployed across multiple programs and, as such, are not separately classified. We use internal resources primarily to conduct our research and discovery and to manage our preclinical development, process development, manufacturing and clinical development activities.

The table below summarizes our direct research and development expenses incurred by program:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
	(in thousands)			
CF program (including MRT5005)	\$ 5,263	\$ 4,570	\$ 11,884	\$ 8,614
OTC deficiency program (including MRT5201)	2,217	2,707	4,087	5,325
MRT discovery program	1,500	1,138	3,543	2,007
Vaccine discovery program	288	—	545	—
Oligonucleotide discovery program	62	10	95	79
Unallocated research and development expenses	7,295	6,794	13,894	11,896
Total research and development expenses	<u>\$ 16,625</u>	<u>\$ 15,219</u>	<u>\$ 34,048</u>	<u>\$ 27,921</u>

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, primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect that our research and development expenses will increase substantially over the next several years as we conduct our clinical trials of MRT5005 for the treatment of patients with CF; conduct a SAD Phase 1/2 clinical trial for MRT5201 in adult patients with OTC deficiency, conduct additional preclinical studies to support our IND for our future clinical development of MRT5201, including a MAD Phase 1/2 clinical trial; conduct research and development activities to advance mRNA vaccines and develop an mRNA vaccine platform under the Sanofi Agreement; prepare regulatory filings for our product candidates; continue to discover and develop additional product candidates; and potentially advance product candidates from our MRT platform into later stages of clinical development. We expect to continue to devote a substantial portion of our resources to our MRT platform for the foreseeable future.

The successful development and commercialization of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any of our product candidates. This uncertainty is due to the numerous risks and uncertainties associated with product development and commercialization, including the uncertainty of:

- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- our ability to maintain our current research and development programs and to establish new ones;
- establishing an appropriate safety profile with IND enabling studies;
- successful patient enrollment in, and the initiation and completion of, clinical trials;
- the successful completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- the receipt of regulatory approvals from applicable regulatory authorities;
- the timing, receipt and terms of any marketing approvals from applicable regulatory authorities;

---

## [Table of Contents](#)

- our ability to establish new licensing or collaboration arrangements;
- the success of our collaboration with Sanofi;
- the performance of our future collaborators, if any;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- development and timely delivery of commercial-grade drug formulations that can be used in our clinical trials and for commercial launch;
- obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights;
- launching commercial sales of our product candidates, if approved, whether alone or in collaboration with others; and
- maintaining a continued acceptable safety profile of the product candidates following approval.

Any changes in the outcome of any of these variables with respect to the development of our product candidates in preclinical and clinical development could mean a significant change in the costs and timing associated with the development of these product candidates. For example, in December 2018, we submitted an IND to the FDA supporting the initiation of a SAD and MAD Phase 1/2 clinical trial for MRT5201 in patients with OTC deficiency. Subsequently, the FDA notified us that our IND for MRT5201 was placed on clinical hold, pending additional preclinical toxicology data. After discussions with the FDA and after we amended the protocol, in June 2019, the FDA removed the clinical hold and allowed us to proceed with a SAD Phase 1/2 clinical trial of MRT5201 in adult patients with OTC deficiency. We are currently conducting additional preclinical studies that are required to support future clinical development of MRT5201, including a MAD clinical trial, and plan to submit data from these studies to the FDA in the fourth quarter of 2019. In addition, if the FDA or another regulatory authority were to delay our planned start of clinical trials or require us to conduct clinical trials or other testing beyond those that we currently expect, or if we experience significant delays in enrollment in any of our planned clinical trials, we could be required to expend significant additional financial resources and time to complete clinical development of that product candidate. We may never obtain regulatory approval for any of our product candidates. Drug commercialization will take several years and millions of dollars in development costs.

### *General and Administrative Expenses*

General and administrative expenses consist primarily of salaries, related benefits and stock-based compensation expense for personnel in executive, finance and administrative functions. General and administrative expenses also include facilities, depreciation and other expenses, which include direct or allocated expenses for rent and maintenance of facilities and insurance, as well as professional fees for legal, patent, consulting, investor and public relations, accounting and audit services.

We anticipate that our general and administrative expenses will increase over the next several years as we anticipate increased accounting, audit, legal, regulatory, compliance, director and officer insurance and investor and public relations costs associated with being a public company.

### *Change in Fair Value of Contingent Consideration*

In connection with our acquisition of the messenger RNA therapeutic platform, or MRT Program, we recognized contingent consideration liabilities for future potential milestone and earnout payment obligations, and prior to the IPO, anti-dilution rights with respect to common stock issued to Shire Human Genetic Therapies, Inc., or Shire, a subsidiary of Takeda Pharmaceutical Company Ltd. The contingent consideration was initially recorded at fair value on the acquisition date and is subsequently remeasured to fair value at each reporting date. Any changes in the fair value of the contingent consideration liabilities are recognized as operating income or expenses.

### ***Other Income (Expense), Net***

#### *Interest Income*

Interest income consists of income recognized in connection with our investments in money market funds and U.S. government agency bonds.

## Table of Contents

### *Other Income (Expense), Net*

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

### *Income Taxes*

We recognized an income tax benefit of \$1.5 million during the three months ended June 30, 2018 and \$0.5 million and \$2.6 million during the six months ended June 30, 2019 and 2018, respectively. There was no income tax benefit recognized during the three months ended June 30, 2019. The income tax benefits recognized during the three months ended June 30, 2018 and the six months ended June 30, 2019 and 2018 resulted from a reduction in the deferred tax liabilities recorded as part of our acquisition of the MRT Program as well as deferred tax assets recorded for net operating losses generated that have an unlimited carryforward period. Net operating losses generated in 2018 and years thereafter can be carried forward indefinitely.

As of December 31, 2018, we had U.S. federal net operating loss carryforwards of \$190.8 million, of which \$122.1 million will, if not utilized, begin to expire in 2031. As of December 31, 2018, we had U.S. state net operating loss carryforwards of \$171.7 million, which will, if not utilized, begin to expire in 2031. As of December 31, 2018, we also had U.S. federal and state research and development tax credit carryforwards of \$5.1 million and \$2.0 million, respectively, which will, if not utilized, begin to expire in 2032 and 2028, respectively, and orphan drug tax credit carryforwards of \$6.6 million, which begin to expire in 2037. We also have state investment tax credit carryforwards of \$0.4 million, which will, if not utilized, begin to expire in 2019. As of December 31, 2018, we recorded a full valuation allowance against our deferred tax assets, except for \$0.8 million related primarily to indefinite-lived net operating loss carryforwards.

### **Results of Operations**

#### *Comparison of the Three Months Ended June 30, 2019 and 2018*

The following table summarizes our results of operations for the three months ended June 30, 2019 and 2018:

	<b>Three Months Ended June 30,</b>		<b>Change</b>
	<b>2019</b>	<b>2018</b>	
	<b>(in thousands)</b>		
Collaboration revenue	\$ 1,174	\$ —	\$ 1,174
Operating expenses:			
Research and development	16,625	15,219	1,406
General and administrative	7,850	5,991	1,859
Change in fair value of contingent consideration	4,889	7,852	(2,963)
Total operating expenses	29,364	29,062	302
Loss from operations	(28,190)	(29,062)	872
Other income (expense):			
Interest income	358	91	267
Other expense	—	(32)	32
Total other income (expense), net	358	59	299
Loss before benefit from income taxes	(27,832)	(29,003)	1,171
Benefit from income taxes	—	1,500	(1,500)
Net loss	\$ (27,832)	\$ (27,503)	\$ (329)

#### *Collaboration Revenue*

Collaboration revenue was \$1.2 million for the three months ended June 30, 2019, which was derived from the Sanofi Agreement. There was no collaboration revenue recognized in the three months ended June 30, 2018.

## Table of Contents

### Research and Development Expenses

	Three Months Ended June 30,		Change
	2019	2018	
	(in thousands)		
Direct external research and development expenses by program:			
CF program (including MRT5005)	\$ 5,263	\$ 4,570	\$ 693
OTC deficiency program (including MRT5201)	2,217	2,707	(490)
MRT discovery program	1,500	1,138	362
Vaccine discovery program	288	—	288
Oligonucleotide discovery program	62	10	52
Unallocated research and development expenses:			
Personnel related (including stock-based compensation)	4,701	4,459	242
Other	2,594	2,335	259
Total research and development expenses	<u>\$ 16,625</u>	<u>\$ 15,219</u>	<u>\$1,406</u>

Research and development expenses were \$16.6 million for the three months ended June 30, 2019, compared to \$15.2 million for the three months ended June 30, 2018. The increase of \$1.4 million was primarily due to increases in external research and development service costs resulting from costs incurred to conduct our Phase 1/2 clinical trial of MRT5005 for the treatment of patients with CF and continued development of our MRT discovery program and our vaccine discovery program.

Direct external expenses of our CF program increased by \$0.7 million in the three months ended June 30, 2019 compared to the three months ended June 30, 2018 primarily due to increased raw material and manufacturing costs as well as increased costs related to our Phase 1/2 clinical trial of MRT5005 for the treatment of patients with CF.

Direct external expenses of our OTC deficiency program decreased by \$0.5 million in the three months ended June 30, 2019 compared to the three months ended June 30, 2018. Expenses incurred in the three months ended June 30, 2018 included increased preclinical development and IND-enabling studies compared to the same period in 2019, partially offset by the initial costs of CROs to conduct our Phase 1/2 clinical trial of MRT5201 for the treatment of patients with OTC deficiency.

Direct external expenses of our MRT discovery program increased by \$0.4 million in the three months ended June 30, 2019 compared to the three months ended June 30, 2018 primarily due to increased costs related to our ongoing exploratory research in the program.

Direct external expenses of our vaccine discovery program increased by \$0.3 million in the three months ended June 30, 2019 compared to the three months ended June 30, 2018 as a result of the Sanofi Agreement which became effective in July 2018. The expenses in the three months ended June 30, 2019 were related to our exploratory research in the program.

Unallocated research and development expenses increased by \$0.5 million in the three months ended June 30, 2019 compared to the three months ended June 30, 2018. The increase of \$0.2 million in personnel-related costs was primarily related to an increase in headcount in the three months ended June 30, 2019 compared to the same period in 2018, partially offset by a decrease in share-based compensation. In the three months ended June 30, 2018, we recorded a fair value adjustment to share-based compensation for non-employees and a stock option modification for which there was no comparable expense in the same period in 2019. The increase of \$0.3 million in other unallocated research and development expenses was due to an increase of \$0.4 million in professional fees related to manufacturing and an increase of \$0.5 million in depreciation and amortization expense related to the definite-lived MRT intangible asset, partially offset by a decrease of \$0.7 million in facilities costs.

### General and Administrative Expenses

General and administrative expenses were \$7.9 million for the three months ended June 30, 2019, compared to \$6.0 million for the three months ended June 30, 2018. The increase of \$1.9 million was primarily due to an increase of \$1.5 million in personnel-related costs, an increase of \$0.3 million in professional fees and an increase of \$0.3 million in insurance costs, all partially offset by a decrease of \$0.4 million in depreciation and amortization expense.

The increase in personnel-related costs was primarily due to an increase in stock-based compensation expense, resulting from options granted during the year ended December 31, 2018 and the six months ended June 30, 2019, as well as an increase in headcount in the three months ended June 30, 2019 compared to the three months ended June 30, 2018.

## [Table of Contents](#)

The increase in professional fees was due to an increase in legal fees primarily associated with filing patent applications and prosecuting our intellectual property portfolio.

The increase in insurance costs was a result of additional insurance coverage associated with operating as a publicly traded company.

The decrease in depreciation and amortization expense was due to the acceleration of unamortized leasehold improvements for a terminated lease in 2018.

### *Change in Fair Value of Contingent Consideration*

In the three months ended June 30, 2019 and 2018, we recognized operating expenses of \$4.9 million and \$7.9 million, respectively, for changes in the fair value of the contingent consideration liabilities we recorded in connection with our acquisition of the MRT Program in December 2016. The contingent consideration liabilities relate to future potential milestone and earnout payment obligations and, prior to the IPO, anti-dilution rights with respect to common stock issued to Shire. The \$4.9 million expense recognized during the three months ended June 30, 2019 was attributed primarily to an increase in the fair value of the contingent consideration liability for future earnout payments that could become due. The increase in the fair value of contingent consideration was primarily due to the continued progress of MRT5005 and MRT5201 and the time value of money due to the passage of time.

### *Benefit from Income Taxes*

During the three months ended June 30, 2018, we recognized an income tax benefit of \$1.5 million. There was no income tax benefit recognized during the three months ended June 30, 2019. The income tax benefit recognized during the three months ended June 30, 2018 resulted from a reduction in the deferred tax liabilities recorded as part of our acquisition of the MRT Program as well as deferred tax assets recorded for net operating losses generated that have an unlimited carryforward period. Net operating losses generated in 2018 and years thereafter can be carried forward indefinitely.

### **Comparison of the Six Months Ended June 30, 2019 and 2018**

The following table summarizes our results of operations for the six months ended June 30, 2019 and 2018:

	<b>Six Months Ended June 30,</b>		<b>Change</b>
	<b>2019</b>	<b>2018</b>	
	<b>(in thousands)</b>		
Collaboration revenue	\$ 2,648	\$ —	\$ 2,648
Operating expenses:			
Research and development	34,048	27,921	6,127
General and administrative	14,403	10,769	3,634
Change in fair value of contingent consideration	16,591	12,760	3,831
Total operating expenses	65,042	51,450	13,592
Loss from operations	\$ (62,394)	(51,450)	(10,944)
Other income (expense):			
Interest income	878	181	697
Other expense	—	(45)	45
Total other income (expense), net	878	136	742
Loss before benefit from income taxes	(61,516)	(51,314)	(10,202)
Benefit from income taxes	486	2,602	(2,116)
Net loss	<u>\$ (61,030)</u>	<u>\$ (48,712)</u>	<u>\$ (12,318)</u>

### *Collaboration Revenue*

Collaboration revenue was \$2.6 million for the six months ended June 30, 2019, which was derived from the Sanofi Agreement. There was no collaboration revenue recognized in the six months ended June 30, 2018.

## Table of Contents

### Research and Development Expenses

	Six Months Ended June 30,		Change
	2019	2018	
	(in thousands)		
Direct external research and development expenses by program:			
CF program (including MRT5005)	\$ 11,884	\$ 8,614	\$ 3,270
OTC deficiency program (including MRT5201)	4,087	5,325	(1,238)
MRT discovery program	3,543	2,007	1,536
Vaccine discovery program	545	—	545
Oligonucleotide discovery program	95	79	16
Unallocated research and development expenses:			
Personnel related (including stock-based compensation)	8,986	7,913	1,073
Other	4,908	3,983	925
Total research and development expenses	<u>\$ 34,048</u>	<u>\$ 27,921</u>	<u>\$ 6,127</u>

Research and development expenses were \$34.0 million for the six months ended June 30, 2019, compared to \$27.9 million for the six months ended June 30, 2018. The increase of \$6.1 million was primarily due to increases in external research and development service costs resulting from costs incurred to conduct our Phase 1/2 clinical trial of MRT5005 for the treatment of patients with CF and continued development of our MRT discovery program, as well as an increase in personnel-related costs.

Direct external expenses of our CF program increased by \$3.3 million in the six months ended June 30, 2019 compared to the six months ended June 30, 2018 primarily due to increased raw material and manufacturing costs as well as increased costs related to our Phase 1/2 clinical trial of MRT5005 for the treatment of patients with CF.

Direct external expenses of our OTC deficiency program decreased by \$1.2 million in the six months ended June 30, 2019 compared to the six months ended June 30, 2018. Expenses incurred in the six months ended June 30, 2018 included increased preclinical development and IND-enabling studies compared to the same period in 2019, partially offset by the initial costs of CROs during the six months ended June 30, 2019 to conduct our Phase 1/2 clinical trial of MRT5201 for the treatment of patients with OTC deficiency.

Direct external expenses of our MRT discovery program increased by \$1.5 million in the six months ended June 30, 2019 compared to the six months ended June 30, 2018 primarily due to increased costs related to our ongoing exploratory research in the program.

Direct external expenses of our vaccine discovery program increased by \$0.5 million in the six months ended June 30, 2019 compared to the six months ended June 30, 2018 as a result of the Sanofi Agreement which became effective in July 2018. The expenses in the six months ended June 30, 2019 were related to our exploratory research in the program.

Unallocated research and development expenses increased by \$2.0 million in the six months ended June 30, 2019 compared to the six months ended June 30, 2018. The increase of \$1.1 million in personnel-related costs was primarily related to an increase in headcount in the six months ended June 30, 2019 compared to the same period in 2018. The increase of \$0.9 million in other unallocated research and development expenses was due to an increase of \$1.0 million in depreciation and amortization expense primarily related to amortization expense recorded in the six months ended June 30, 2019 related to the definite-lived MRT intangible asset, an increase of \$0.4 million related to maintenance and license fees and an increase of \$0.3 million in professional fees primarily related to manufacturing, partially offset by a decrease of \$0.9 million in facilities costs.

### General and Administrative Expenses

General and administrative expenses were \$14.4 million for the six months ended June 30, 2019, compared to \$10.8 million for the six months ended June 30, 2018. The increase of \$3.6 million was primarily due to an increase of \$2.7 million in personnel-related costs, an increase of \$0.6 million in professional fees and an increase of \$0.6 million in insurance costs, all offset by a decrease of \$0.5 million in depreciation and amortization expense.

The increase in personnel-related costs was primarily due to an increase in stock-based compensation expense, resulting from options granted during the year ended December 31, 2018 and the six months ended June 30, 2019, as well as an increase in headcount in the six months ended June 30, 2019 compared to the six months ended June 30, 2018.

---

## [Table of Contents](#)

The increase in professional fees was due to an increase in legal fees primarily associated with filing patent applications and prosecuting our intellectual property portfolio.

The increase in insurance costs was a result of additional insurance coverage associated with operating as a publicly traded company.

The decrease in depreciation and amortization expense was primarily due to the acceleration of unamortized leasehold improvements for a terminated lease in 2018.

### *Change in Fair Value of Contingent Consideration*

In the six months ended June 30, 2019 and 2018, we recognized operating expenses of \$16.6 million and \$12.8 million, respectively, for changes in the fair value of the contingent consideration liabilities we recorded in connection with our acquisition of the MRT Program in December 2016. The contingent consideration liabilities relate to future potential milestone and earnout payment obligations and, prior to the IPO, anti-dilution rights with respect to common stock issued to Shire. The \$16.6 million expense recognized during the six months ended June 30, 2019 was attributed primarily to an increase in the fair value of the contingent consideration liability for future earnout payments that could become due. The increase in the fair value of contingent consideration was primarily due to the continued progress of MRT5005 and MRT5201, the time value of money due to the passage of time and a decrease in the discount rate.

### *Benefit from Income Taxes*

During the six months ended June 30, 2019 and 2018, we recognized an income tax benefit of \$0.5 million and \$2.6 million, respectively. The income tax benefits recognized during the six months ended June 30, 2019 and 2018 resulted from a reduction in the deferred tax liabilities recorded as part of our acquisition of the MRT Program as well as deferred tax assets recorded for net operating losses generated that have an unlimited carryforward period. Net operating losses generated in 2018 and years thereafter can be carried forward indefinitely.

## **Liquidity and Capital Resources**

Since our inception, we have not generated any revenue from product sales, have generated only limited revenue from the Sanofi Agreement and have incurred significant operating losses and negative cash flows from our operations. 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. See “—Funding Requirements” and Note 1 to the consolidated financial statements appearing in this Quarterly Report on Form 10-Q for a further discussion of our liquidity and the conditions and events that raise substantial doubt regarding our ability to continue as a going concern.

Through June 30, 2019, we have funded our operations primarily with net cash proceeds of \$189.2 million from the sale of redeemable convertible preferred stock and the sale of bridge units, which ultimately converted into shares of our common stock, net cash proceeds of \$113.2 million from our IPO, \$45.0 million from the upfront payment received under the Sanofi Agreement and net cash proceeds of \$44.1 million from a private placement of our common stock.

## [Table of Contents](#)

On July 2, 2018, we closed our IPO in which we issued and sold 9,714,371 shares of common stock, including the underwriters' over-allotment option, at a public offering price of \$13.00 per share, resulting in aggregate net proceeds of \$113.2 million after deducting underwriting discounts and commissions and offering expenses.

On May 3, 2019, we issued and sold 5,582,940 shares of our common stock in a private placement at a price per share of \$8.50, resulting in gross proceeds of \$47.5 million, before deducting placement agent fees of \$2.8 million and other offering expenses of \$0.6 million.

On July 3, 2019, we filed a universal shelf registration statement on Form S-3 with the SEC, or the 2019 Shelf, to register for sale from time to time up to \$250.0 million of common stock, preferred stock, debt securities, warrants and/or units in one or more offerings, which became effective on July 19, 2019 (File No. 333-232543).

On July 3, 2019, we entered into an Open Market Sale Agreement<sup>SM</sup>, or Sales Agreement, with Jefferies LLC, or Jefferies, under which we may issue and sell shares of common stock, from time to time, having an aggregate offering price of up to \$50.0 million. We have not sold any shares under the Sales Agreement as of July 29, 2019. Sales of common stock through Jefferies may be made by any method that is deemed an "at the market" offering as defined in Rule 415 promulgated under the Securities Act of 1933, as amended. Jefferies has agreed to use its commercially reasonable efforts consistent with its normal trading and sales practices to sell our shares of common stock based upon our instructions. We are not obligated to make any sales of our common stock under the Sales Agreement. Any shares sold would be pursuant to the 2019 Shelf.

### **Cash Flows**

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

	<b>Six Months Ended June 30,</b>	
	<b>2019</b>	<b>2018</b>
	<b>(in thousands)</b>	
Net cash used in operating activities	\$ (41,445)	\$ (33,581)
Net cash provided by investing activities	15,525	4,161
Net cash provided by (used in) financing activities	45,550	(2,143)
Net increase (decrease) in cash, cash equivalents and restricted cash	<u>\$ 19,630</u>	<u>\$ (31,563)</u>

#### *Operating Activities*

During the six months ended June 30, 2019, operating activities used \$41.4 million of cash, resulting from our net loss of \$61.0 million and net cash used by changes in our operating assets and liabilities of \$4.0 million, partially offset by net non-cash charges of \$23.6 million. Net cash used by changes in our operating assets and liabilities consisted of a \$1.9 million decrease in accounts payable and a \$1.8 million increase in prepaid expenses and other assets. Net non-cash charges for the six months ended June 30, 2019 primarily consisted of a \$16.6 million increase in the change in the fair value of contingent consideration which was primarily due to the continued progress of MRT5005 and MRT5201, the time value of money due to the passage of time and a decrease in the discount rate.

During the six months ended June 30, 2018, operating activities used \$33.6 million of cash, resulting from our net loss of \$48.7 million and net cash used by changes in our operating assets and liabilities of \$0.8 million, partially offset by net non-cash charges of \$15.9 million. Net cash used by changes in our operating assets and liabilities for the six months ended June 30, 2018 consisted of a \$2.5 million increase in prepaid expenses, partially offset by a \$1.1 million increase in accounts payable and a \$0.4 million increase in deferred rent.

#### *Investing Activities*

During the six months ended June 30, 2019, investing activities provided \$15.5 million of cash, consisting of \$55.8 million of sales and maturities of short-term investments, partially offset by \$38.4 million of purchases of short-term investments and \$1.8 million of purchases of property and equipment.

During the six months ended June 30, 2018, net cash provided by investing activities was \$4.2 million, consisting of \$14.9 million of sales and maturities of short-term investments, partially offset by \$6.0 million of purchases of short-term investments as well as \$4.8 million of purchases of property and equipment, which primarily consisted of leasehold improvements and other property related to our lease of office and laboratory space in Lexington, Massachusetts.

---

## [Table of Contents](#)

### *Financing Activities*

During the six months ended June 30, 2019, net cash provided by financing activities was \$45.6 million, consisting of net cash proceeds of \$44.1 million from a private placement of our common stock and \$1.4 million in proceeds from option exercises.

During the six months ended June 30, 2018, net cash used in financing activities was \$2.1 million, consisting of \$2.4 million in payments of initial public offering costs, partially offset by \$0.3 million of proceeds from option exercises.

### *Funding Requirements*

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, continue ongoing and initiate new clinical trials of and seek marketing approval for our product candidates. In addition, we expect to incur additional costs associated with operating as a public company. Our expenses will also increase if, and as, we:

- leverage our programs to advance our other product candidates into preclinical and clinical development;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- seek to discover and develop additional product candidates;
- establish a sales, marketing, medical affairs and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval and intend to commercialize on our own or jointly;
- hire additional clinical, quality control and scientific personnel;
- expand our operational, financial and management systems and increase personnel, including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;
- maintain, expand and protect our intellectual property portfolio; and
- acquire or in-license other product candidates and technologies.

We believe that our existing cash, cash equivalents and short-term investments of \$146.9 million as of June 30, 2019 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2020. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. In accordance with the requirements of ASC 205-40, based on our recurring losses and cash outflows from operations since inception, expectation of continuing operating losses and cash outflows from operations for the foreseeable future and the need to raise additional capital to finance our future operations, we have concluded that there is substantial doubt about our ability to continue as a going concern. The financial statements do not include any adjustments that might result from the outcome of this uncertainty. There is no assurance that we will be successful in obtaining additional financing on terms acceptable to us, if at all, nor is it considered probable under the accounting standards. As such, under the requirements of ASC 205-40, management may not consider the potential for future capital raises or management plans to reduce costs that are not considered probable in their assessment of our ability to meet our obligations.

We will need to raise additional capital or incur indebtedness to continue to fund our operations in the future. Our ability to raise additional funds will depend on financial, economic and market conditions, many of which are outside of our control, and we may be unable to raise financing when needed, or on terms favorable to us. If we are unable to raise additional funds when needed, we may be required to delay, reduce or eliminate our product development or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves, which could adversely affect our business prospects, and we may be unable to continue our operations. Because of the numerous risks and uncertainties associated with research, development and commercialization of product candidates, we are unable to estimate the exact amount of our working capital requirements. Factors that may affect our planned future capital requirements and accelerate our need for additional working capital include the following:

- the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical studies and clinical trials;
- the costs, timing and outcome of regulatory review of our product candidates;

---

## [Table of Contents](#)

- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- the costs of manufacturing commercial-grade products and sufficient inventory to support commercial launch;
- the ability to receive additional non-dilutive funding, including grants from organizations and foundations;
- the revenue, if any, received from commercial sale of our products, should any of our product candidates receive marketing approval;
- the cost and timing of hiring new employees to support our continued growth;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the ability to establish and maintain collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies;
- the timing, receipt and amount of sales of, or milestone payments related to or royalties on, our current or future product candidates, if any; and
- our ability to continue as a going concern.

A change in the outcome of any of these or other variables with respect to the development of any of our product candidates could significantly change the costs and timing associated with the development of that product candidate. Further, our operating plans may change in the future, and we may need additional funds to meet operational needs and capital requirements associated with such operating plans.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic partnerships or marketing, distribution or licensing arrangements with third parties and grants from organizations and foundations. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of our common stockholders may be materially diluted, and the terms of such securities could include liquidation or other preferences that could adversely affect the rights of our common stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include restrictive covenants that limit our ability to take specified actions, such as incurring additional debt, making capital expenditures or declaring dividends. In addition, debt financing would result in increased fixed payment obligations.

### **Contractual Obligations and Commitments**

During the six months ended June 30, 2019, there were no material changes to our contractual obligations and commitments as of December 31, 2018 described under Management's Discussion and Analysis of Financial Condition and Results of Operations in our 2018 Annual Report.

### **Critical Accounting Policies and Significant Judgments and Estimates**

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which we have prepared in accordance with generally accepted accounting principles in the United States. We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate, and different estimates—which also would have been reasonable—could have been used. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Our actual results may differ from these estimates under different assumptions or conditions.

---

## [Table of Contents](#)

There have been no material changes to our critical accounting policies from those described in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in the 2018 Annual Report except for the adoption of Accounting Standards Update No. 2016 02, Leases (Topic 842), or ASC 842, on January 1, 2019 as discussed below.

We adopted ASC 842 as of the required effective date of January 1, 2019. Under ASC 842, most leases are required to be recognized on the balance sheet as a right-of-use asset and a lease liability. The standard has been implemented using the cumulative-effect adjustment on the effective date of the standard, with comparative periods presented in accordance with the previous guidance. We have also elected to utilize the permitted practical expedients, which allowed us to not reassess previous accounting conclusions around whether arrangements are or contain leases, and carried forward both the historical classification of leases and the treatment of initial direct costs. ASC 842 requires us to make significant assumptions and judgments including, but not limited to, the determination of whether a contract contains a lease, the allocation of consideration in a contract between lease and non-lease components and the determination of the discount rate for the lease. Our assessment of leases under ASC 842 is based upon assumptions we believed to be reasonable, but which are inherently uncertain, and changes in these assumptions could materially affect the amounts recognized as a right-of-use asset and lease liability on the balance sheet, and, as a result, actual results may differ materially from estimates. The adoption of this standard did not materially impact our consolidated net earnings and had no impact on cash flows.

### **Emerging Growth Company Status**

The Jumpstart Our Business Startups Act of 2012 permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have irrevocably elected to “opt out” of this provision and, as a result, we will comply with new or revised accounting standards when they are required to be adopted by public companies that are not emerging growth companies.

### **Off-Balance Sheet Arrangements**

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

### **Item 3. Quantitative and Qualitative Disclosures About Market Risk.**

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*

We maintain “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, that are designed to ensure that information required to be disclosed in the reports we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. In designing and evaluating our disclosure controls and procedures, management recognizes that any 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 benefits of possible controls and procedures relative to their costs.

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, who serve as our principal executive officer and principal financial and accounting officer, respectively, has evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2019. Based on such evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of June 30, 2019.

---

[Table of Contents](#)

***Changes in Internal Control over Financial Reporting***

There have been no changes in internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the three months ended June 30, 2019 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II—OTHER INFORMATION

### Item 1. Legal Proceedings.

We are not currently subject to any material legal proceedings.

### Item 1A. Risk Factors.

*Investing in our common stock involves a high degree of risk. Before investing in our common stock, you should consider carefully the risks described below, together with the other information contained in this Quarterly Report on Form 10-Q, including our financial statements and the related notes and in our other filings with the Securities and Exchange Commission, or SEC. If any of the following risks occur, our business, financial condition, results of operations and prospects could be materially and adversely affected. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.*

#### Risks Related to our Financial Position and Need for Additional Capital

***We have incurred significant losses since inception. We expect to incur losses for at least the next several years and may never achieve or maintain profitability.***

Since inception, we have incurred significant losses. Our net loss was \$61.0 million and \$97.4 million for the six months ended June 30, 2019 and for the year ended December 31, 2018, respectively. As of June 30, 2019, we had an accumulated deficit of \$307.2 million. As noted below, we and our auditors have identified conditions and events that raise substantial doubt about our ability to continue as a going concern. We have funded our operations to date primarily with proceeds from the sale of redeemable convertible preferred stock and the sale of bridge units, which ultimately converted into shares of our common stock, the proceeds from our IPO, an upfront payment received under the Sanofi Agreement and the proceeds from a private placement of our common stock. We expect that it could be several years, if ever, before we have a commercialized product candidate. We expect to continue to incur significant expenses and operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we:

- continue the clinical development of MRT5005 and MRT5201;
- leverage our programs to advance our other product candidates into preclinical and clinical development;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- seek to discover and develop additional product candidates;
- establish a sales force, marketing, medical affairs and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval and intend to commercialize on our own or jointly;
- hire additional clinical, quality control and scientific personnel;
- expand our operational, financial and management systems and increase personnel, including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;
- maintain, expand and protect our intellectual property portfolio;
- acquire or in-license other product candidates and technologies; and
- incur additional legal, accounting and other expenses in operating as a public company.

To become and remain profitable, we, or our collaborators, must develop and eventually commercialize product candidates with significant market potential. This will require us to succeed in a range of challenging activities, including completing preclinical studies and clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we may obtain marketing approval and satisfying any post-marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate sufficient revenue to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

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

We have never generated revenue from product sales. Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with our collaborative partners, to successfully develop and obtain the regulatory approvals necessary to commercialize our product candidates. We do not have any products approved for sale and do not anticipate generating revenue from product sales for the next several years, if ever. Our ability to generate future revenue from product sales depends heavily on our, or our collaborators', success in:

- completing preclinical and clinical development of our product candidates and identifying and developing new product candidates;
- seeking and obtaining marketing approvals for any of our product candidates;
- launching and commercializing product candidates for which we obtain marketing approval by establishing a sales force, marketing, medical affairs and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- achieving formulary status in hospitals and adequate coverage and reimbursement by government and third-party payors for our product candidates;
- establishing and maintaining 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 our product candidates, if approved;
- obtaining market acceptance of our product candidates as viable treatment options;
- addressing any competing technological and market developments;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such collaborations;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;
- defending against third-party interference or infringement claims, if any; and
- attracting, hiring and retaining qualified personnel.

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

---

## [Table of Contents](#)

### ***Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.***

Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring or discovering product candidates and securing related intellectual property rights, conducting discovery, research and development activities for our programs, undertaking preclinical studies, entering into licensing agreements and planning for potential commercialization. While we are conducting a Phase 1/2 clinical trial of MRT5005, initiating a single-ascending dose, or SAD, Phase 1/2 clinical trial of MRT5201, and conducting additional preclinical studies to support future clinical development of MRT5201, including a multiple-ascending dose, or MAD, Phase 1/2 clinical trial, we have not yet completed a clinical trial of any of our product candidates. We have not yet demonstrated the ability to obtain marketing approvals, manufacture a commercial-scale product or conduct sales and marketing activities necessary for successful commercialization. Consequently, any evaluation of our business to date or predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

If we obtain marketing approval for any of our product candidates, we will need to transition from a company with a research and development focus to a company capable of supporting commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays and may not be successful in such a transition.

### ***We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain capital when needed may force us to delay, reduce or eliminate certain of our product development efforts or other operations.***

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, continue ongoing and initiate clinical trials of and seek marketing approval for our product candidates. These expenditures will include costs associated with our asset purchase agreement, as amended, with Shire Human Genetic Therapies, Inc., or Shire, a subsidiary of Takeda Pharmaceutical Company Ltd., or the Shire Agreement. Under the terms of the Shire Agreement, we are obligated to make significant cash payments upon the achievement of specified commercial milestones, as well as earnout payments in connection with sales of products based on the compounds that we acquired from Shire.

We will require additional capital to advance MRT5005 and MRT5201 and any other product candidates we develop through necessary clinical trials and clinical development. In addition, if we obtain marketing approval for any of our product candidates that we plan to commercialize ourselves, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. Furthermore, we expect to continue to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain additional funding in connection with our continuing operations. We may raise this additional funding through the sale of equity, debt financings or other capital sources, including potential collaborations with other companies or other strategic transactions and funding under government or other contracts. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans.

We believe that our existing cash, cash equivalents and short-term investments of \$146.9 million as of June 30, 2019 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2020. As of June 30, 2019, management has further assessed this risk and, in accordance with the requirements of Accounting Standards Update No. 2014-15, Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40), or ASC 205-40, determined that there is substantial doubt about our ability to continue as a going concern. There is no assurance that we will be successful in obtaining additional financing on terms acceptable to us, if at all, nor is it considered probable under the accounting standards. As such, under the requirements of ASC 205-40, management may not consider the potential for future capital raises or management plans to reduce costs that are not considered probable in their assessment of our ability to meet our obligations. If we are unable to obtain funding, we may be required to delay, reduce or eliminate our product development or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves, which could adversely affect our business prospects, and we may be unable to continue operations. To finance our operations beyond that point, we will need to raise additional capital, which cannot be assured.

Furthermore, our future funding requirements will depend on, and could increase significantly as a result of, many factors, including:

- the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical studies and clinical trials;
- the costs, timing and outcome of regulatory review of our product candidates;

---

## Table of Contents

- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- the costs of manufacturing commercial-grade products and sufficient inventory to support commercial launch;
- the success of our collaboration with Sanofi;
- the ability to receive additional non-dilutive funding, including grants from organizations and foundations;
- the revenue, if any, received from commercial sale of our products, should any of our product candidates receive marketing approval;
- the cost and timing of hiring new employees to support our continued growth;
- costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies;
- the timing, receipt and amount of sales of, or milestone payments related to or royalties on, our current or future product candidates, if any; and
- our ability to continue as a going concern.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that typically takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenue, if any, and any commercial milestones or royalty payments under any collaboration agreements that we enter into, including our collaboration with Sanofi, will be derived from or based on sales of products that may not be commercially available for many years, if at all. Accordingly, we will continue to rely on additional financing to achieve our business objectives.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. We cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. Our issuance of additional securities, whether equity or debt, or the possibility of such issuance, may cause the market price of our common stock to decline, and our stockholders may not agree with our financing plans or the terms of such financings.

Our failure to raise capital as and when needed would negatively impact our financial condition and our ability to pursue our business strategy, and we could be forced to delay, reduce or eliminate certain of our research and development programs or any future commercialization efforts.

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

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through the combination of public or private equity offerings, debt financings, grants, collaborations, strategic partnerships or marketing, distribution or licensing arrangements with third parties. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest may be materially diluted, and the terms of such securities could include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include restrictive covenants that limit our ability to take specified actions, such as incurring debt, making capital expenditures or declaring dividends. In addition, debt financing would result in increased fixed payment obligations.

If we raise funds through collaborations, strategic partnerships or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds when needed, we will be required to delay, reduce or eliminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

---

## [Table of Contents](#)

### ***We have identified conditions and events that raise substantial doubt about our ability to continue as a going concern.***

As of June 30, 2019, we had \$146.9 million in existing cash, cash equivalents and short-term investments. We expect these available cash resources to fund our operating expenses and capital expenditure requirements into the second half of 2020. Based on our available cash resources, we do not have sufficient cash on hand to support current operations for at least the next twelve months from the date of filing this Quarterly Report on Form 10-Q. If we are unable to obtain sufficient funding, we may be forced to delay or reduce the scope of our development programs, our business, prospects, financial condition and results of operations will be materially and adversely affected, and we may be unable to continue as a going concern. If we are unable to continue as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our audited financial statements, and it is likely that investors will lose all or a part of their investment. Future reports from our independent registered public accounting firm may contain statements expressing substantial doubt about our ability to continue as a going concern. If we seek additional financing to fund our business activities in the future and there remains substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide funding to us on commercially reasonable terms, if at all.

### ***We may be required to make payments in connection with our acquisition of the MRT Program from Shire.***

In December 2016, we acquired the messenger RNA, or mRNA, therapeutic platform, or MRT Program, pursuant to the Shire Agreement. Under the Shire Agreement, we are obligated to make milestone payments to Shire of up to \$60.0 million in the aggregate upon the occurrence of specified commercial milestones, including upon the first commercial sale of a product that includes or is composed of MRT compounds acquired from Shire, or MRT Product, for the treatment of cystic fibrosis, or CF, and upon the achievement of a specified level of annual net sales with respect to MRT Products. We are also obligated to make additional milestone payments of \$10.0 million for each non-CF MRT Product upon the first commercial sale of a non-CF MRT Product; provided that such milestone payments will only be due once for any two non-CF MRT Products that contain the same MRT compounds or once per non-CF MRT Product that is a vaccine developed under our collaboration with Sanofi. Under the Shire Agreement, we are also obligated to pay a fixed, quarterly earnout payment of a mid-single-digit percentage of net sales of each MRT Product. The earnout period will begin on the date of the first commercial sale of MRT Products and will end, on a product-by-product and country-by-country basis, on the later of (1) the expiration of the last valid claim of the assigned patents covering the manufacture, use or composition of such product in such country of the applicable MRT Product and (2) 10 years after the first commercial sale of the MRT Product in such country. If these payments become due under the terms of the Shire Agreement, we may not have sufficient funds available to meet our obligations and our development efforts may be materially harmed. If a combination MRT Product that is a vaccine is sold, in certain circumstances, we would be obligated to pay Shire a royalty on a minimum portion of net sales.

### ***Comprehensive tax reform legislation could adversely affect our business and financial condition.***

On December 22, 2017, the U.S. government enacted the Tax Cuts and Jobs Act, or the Tax Act, which significantly reformed the Internal Revenue Code of 1986, as amended, or the Code. The Tax Act contains, among other things, significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for net interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses to 80% of annual taxable income and elimination of net operating loss carrybacks, one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the Tax Act is uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain how various states will respond to the Tax Act. The impact of this tax reform on holders of our common stock is also uncertain and could be adverse. We urge prospective investors in our common stock to consult with their legal and tax advisors with respect to this legislation and the potential tax consequences of investing in or holding our common stock.

---

## [Table of Contents](#)

### ***We might not be able to utilize a significant portion of our net operating loss carryforwards and research and development tax credit carryforwards.***

As of December 31, 2018, we had federal net operating loss carryforwards of \$190.8 million, of which \$122.1 million will, if not utilized, begin to expire in 2031. As of December 31, 2018, we had state net operating loss carryforwards of \$171.7 million, which will, if not utilized, begin to expire in 2031. Our federal and state research and development tax credit carryforwards of \$5.1 million and \$2.0 million, respectively, will, if not utilized, begin to expire in 2032 and 2028, respectively, and orphan drug tax credit carryforwards of \$6.6 million will, if not utilized, begin to expire in 2037. We also have state investment tax credit carryforwards of \$0.4 million, which will, if not utilized, begin to expire in 2019. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset our future income tax liabilities. Under federal income tax law, federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such carryforwards is limited to 80% of our taxable income in the year in which such carryforwards are used.

In addition, under Section 382 of the Code and corresponding provisions of state law, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation’s ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. We have not determined if we have experienced Section 382 ownership changes in the past and if a portion of our net operating loss and tax credit carryforwards are subject to an annual limitation under Section 382. In addition, we may experience ownership changes in the future as a result of subsequent changes in our stock ownership, some of which may be outside of our control. If we determine that an ownership change has occurred and our ability to use our historical net operating loss and tax credit carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations.

### **Risks Related to the Development of Our Product Candidates**

#### ***Our approach to the discovery and development of product candidates based on mRNA is unproven, and we do not know whether we will be able to successfully develop any products.***

We focus on delivering mRNA encoding functional versions of proteins into cells without altering the underlying DNA. Our future success depends on the successful development of this novel therapeutic approach. Relatively few mRNA-based therapeutic product candidates have been tested in animals or humans, and the data underlying the feasibility of developing mRNA-based therapeutic products is both preliminary and limited. To date, no product that utilizes mRNA as a therapeutic has been approved in the United States or Europe. We have not yet succeeded and may not succeed in demonstrating the efficacy and safety of any of our product candidates in clinical trials or in obtaining marketing approval thereafter. We have not yet completed a clinical trial of any product candidate and we have not yet assessed safety of any product candidate in humans. As such, there may be adverse effects from treatment with any of our current or future product candidates that we cannot predict at this time.

As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our MRT platform, or any similar or competitive mRNA platforms, will result in the development and regulatory approval of any products. There can be no assurance that any development problems we experience in the future related to our MRT platform or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Any of these factors may prevent us from completing our preclinical studies or any clinical trials that we may initiate or commercializing any product candidates we may develop on a timely or profitable basis, if at all.

#### ***We have never obtained marketing approval for a product candidate, and we may be unable to obtain, or may be delayed in obtaining, marketing approval for any of our product candidates.***

We are a clinical-stage company and have not received approval from the FDA, EMA or other regulatory authority to market any product candidate. The regulatory review process may be more expensive or take longer than we expect, and we may be required to conduct additional studies and/or trials beyond those we anticipate. If it takes us longer to develop and/or obtain regulatory approval for our product candidates than we expect, such delays could materially and adversely affect our business, financial condition, results of operations and prospects.

---

## [Table of Contents](#)

***We have not yet received clearance from the FDA to proceed with a MAD Phase 1/2 clinical trial of MRT5201 and the FDA is requiring us to submit additional information before we may be permitted to initiate this trial. If we do not receive clearance from the FDA we will not be able to initiate our MAD clinical trial of MRT5201.***

In December 2018, we submitted to the FDA an IND to initiate a SAD and MAD Phase 1/2 clinical trial of MRT5201. This trial is not yet active at any investigational site and has not yet recruited any subjects. In January 2019, the FDA placed the IND on clinical hold, pending additional preclinical toxicology data. After discussions with the FDA and after we amended the protocol in June 2019, we received clearance from the FDA to proceed with a SAD Phase 1/2 clinical trial of MRT5201 in adult patients with OTC deficiency. However, in order to proceed with future clinical development of MRT5201, including a MAD clinical trial, the FDA has requested additional preclinical toxicology data to assess the potential for adverse effects related to the clearance time of MRT5201. We are currently conducting additional preclinical studies that are required to support future clinical development of MRT5201, including a MAD clinical trial, and plan to submit data from these studies to the FDA in the fourth quarter of 2019. If we are unable to complete these studies satisfactorily and submit a regulatory application, we may be unable to initiate a MAD Phase 1/2 clinical trial of MRT5201. Any delay in our ability, or our inability, to receive clearance from the FDA to initiate a MAD Phase 1/2 clinical trial of MRT5201 will delay or terminate our clinical development plans for MRT5201, may require us to incur additional clinical development costs and could impair our ability to ultimately obtain FDA approval for MRT5201. Delays in the completion of any clinical trial of MRT5201 could increase our costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue.

***In the near term, we are dependent on the success of MRT5005 and MRT5201. If we are unable to complete satisfactorily the clinical development of, obtain marketing approval for or successfully commercialize MRT5005 and MRT5201, either alone or with a future collaborator, or if we experience significant delays in doing so, our business would be substantially harmed.***

We do not currently have products approved for sale and are investing a significant portion of our efforts and financial resources in the development of MRT5005 and MRT5201. Our prospects are substantially dependent on our ability, or that of any future collaborator, to develop and obtain marketing approval for, and successfully commercialize, MRT5005 and MRT5201 in one or more disease indications.

The success of MRT5005 and MRT5201 will depend on several factors, including the following:

- successful initiation of clinical trials, including clearance by the FDA to begin a MAD Phase 1/2 clinical trial of MRT5201;
- successful patient enrollment in and completion of clinical trials;
- a safety, tolerability and efficacy profile that is satisfactory to the FDA, EMA or other regulatory authorities for marketing approval;
- timely receipt of marketing approvals from applicable regulatory authorities;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishment and maintenance of arrangements with third-party manufacturers for both clinical and any future commercial manufacturing;
- adequate ongoing availability of raw materials and drug product for clinical development and any commercial sales;
- obtaining and maintaining patent, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protection of our rights in our intellectual property portfolio;
- successful launch of commercial sales following any marketing approval;
- a continued acceptable safety profile following any marketing approval;
- commercial acceptance by hospitals, the patient community, the medical community and third-party payors;
- the availability of coverage and adequate reimbursement from third-party payors;
- the performance of our future collaborators, if any; and
- our ability to compete with other therapies.

---

## [Table of Contents](#)

Many of these factors are beyond our control, including clinical development, whether or not the FDA provides us with clearance to begin a MAD Phase 1/2 clinical trial of MRT5201, the regulatory review process, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of any future collaborator. If we are unable to develop, receive marketing approval for and successfully commercialize MRT5005 and MRT5201, on our own or with any future collaborator, or experience delays as a result of any of these factors or otherwise, our business would be substantially harmed.

***Clinical drug development is a lengthy and expensive process with uncertain timelines and uncertain outcomes. If the initiation or completion of clinical trials of our product candidates, particularly MRT5005 and MRT5201, is prolonged or delayed, we or any future collaborators may be unable to obtain required regulatory approvals, and therefore will be unable to commercialize our product candidates on a timely basis or at all, which will adversely affect our business.***

Before obtaining marketing approval for our product candidates, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates. Clinical testing is expensive, time-consuming, difficult to design and implement and uncertain as to outcome. We cannot guarantee that our clinical trials, such as our Phase 1/2 clinical trial of MRT5005 in patients with CF or our SAD Phase 1/2 clinical trial of MRT5201, will be conducted as planned, completed on schedule, if at all, or yield positive results.

A clinical trial failure can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities or collaborators on trial design;
- delays in successfully addressing the specific information requests made by the FDA with respect to a MAD Phase 1/2 clinical trial of MRT5201;
- delays in reaching agreement on acceptable terms with contract research organizations, or CROs, and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board or independent ethics committee approval at each clinical trial site;
- delays in recruiting suitable subjects or a sufficient number of subjects to participate in our clinical trials;
- imposition of a clinical hold by regulatory authorities, including upon submission of an IND, such as the clinical hold that the FDA had placed on the IND for our Phase 1/2 clinical trial of MRT5201 in January 2019 and subsequently lifted in June 2019 after we amended the protocol, allowing us to proceed with a SAD clinical trial, and the clinical hold that the FDA had placed on the IND for our Phase 1/2 clinical trial of MRT5005 in January 2018, and subsequently lifted by the FDA in April 2018, or as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites;
- failure by us, any CROs we engage, clinical investigators or any other third parties to adhere to clinical trial requirements;
- failure to perform the clinical trial in accordance with good clinical practices, or GCP, or applicable regulatory requirements in the European Union, the United States, or other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- delays or failures in demonstrating the comparability of product manufactured at one facility or with one process to product manufactured at another facility or with another process, including clinical trials to demonstrate such comparability;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or subjects dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors; and
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

---

## [Table of Contents](#)

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenue from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional trials to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

We could encounter delays if a clinical trial is suspended or terminated by us, by the institutional review boards of the institutions in which such trials are conducted or their ethics committees, by the Data Review Committee or Data Safety Monitoring Board for such trial or by the FDA or other foreign regulatory authorities. Such authorities may suspend or terminate a clinical trial 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 foreign regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, including those relating to the class of products to which our product candidates belong.

Any of these occurrences may harm our business, financial condition and prospects significantly. 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 or early termination of the development of our product candidates.

***Preclinical drug development is uncertain. Some or all of our preclinical programs may experience delays or may never advance to clinical trials, which would adversely affect our ability to obtain regulatory approvals or commercialize these product candidates on a timely basis or at all, which would have an adverse effect on our business.***

In order to obtain FDA approval to market a new biological product, we must demonstrate proof of safety, purity and potency or efficacy in humans. To satisfy these requirements, we will have to conduct adequate and well-controlled clinical trials. Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support an IND in the United States. We cannot be certain of the timely completion or outcome of our preclinical testing and studies, and we cannot predict if the FDA will accept our proposed clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of these product candidates. As a result, we cannot be sure that we will be able to submit INDs or similar applications for any preclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of INDs or similar applications will result in the FDA or other regulatory authorities allowing clinical trials to begin. For example, in December 2018, we submitted an IND to the FDA supporting the initiation of a Phase 1/2 clinical trial for MRT5201 in patients with OTC deficiency. Subsequently, the FDA notified us that our IND for MRT5201 was placed on clinical hold. While we have received clearance from the FDA to proceed with a SAD Phase 1/2 clinical trial of MRT5201 in adult patients with OTC deficiency, we must satisfactorily complete additional preclinical studies in support of our IND in order to proceed with a MAD Phase 1/2 clinical trial of MRT5201. We cannot be certain that the data obtained and submitted to the FDA will be accepted and that a MAD Phase 1/2 clinical trial for MRT5201 will be allowed to begin. In addition, after we submitted an IND for MRT5005 to initiate our Phase 1/2 clinical trial in patients with CF, the FDA placed a clinical hold on the IND, requiring us to submit, prior to initiating the trial, additional chemistry, manufacturing and controls information relating to materials and processes used during the manufacture of the product candidate. The FDA lifted the clinical hold for our Phase 1/2 clinical trial of MRT5005 in April 2018.

Conducting preclinical testing is a lengthy, time-consuming and expensive process. The length of time may vary substantially according to the type, complexity, novelty and intended use of the product candidate, and often can be several years or more per product candidate. Delays associated with product candidates for which we are conducting preclinical testing and studies ourselves may cause us to incur additional operating expenses. Moreover, we may be affected by delays associated with the preclinical testing and studies of certain product candidates conducted by our potential partners over which we have no control. The commencement and rate of completion of preclinical studies and clinical trials for a product candidate may be delayed by many factors, including, for example:

- inability to generate sufficient preclinical or other *in vivo* or *in vitro* data to support the initiation of clinical trials; and
- delays in reaching a consensus with regulatory agencies on study design.

---

## [Table of Contents](#)

Moreover, even if we do initiate clinical trials for other product candidates, our development efforts may not be successful, and clinical trials that we conduct or that third parties conduct on our behalf may not demonstrate sufficient safety, purity and potency or efficacy necessary to obtain the requisite regulatory approvals for any of our product candidates or product candidates employing our technology. Even if we obtain positive results from preclinical studies or initial clinical trials, we may not achieve the same success in future trials.

***Success in preclinical studies or early clinical trials may not be indicative of results obtained in later trials.***

Results from preclinical studies are not necessarily predictive of clinical trial results, results from early clinical trials are not necessarily predictive of later clinical trial results and interim results of a clinical trial are not necessarily indicative of final results. Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or successful advancement through initial clinical trials.

There can be no assurance that the success we achieved in preclinical studies of MRT5005 or MRT5201 or may achieve in preclinical studies of other product candidates will result in success in clinical trials of these product candidates. In addition, we cannot assure you that we will be able to achieve the same or similar success in our preclinical studies and clinical trials of our other product candidates.

For example, our preclinical studies in animal models have been conducted using human mRNA, which differs from animal mRNA, making it difficult for us to use animal models to assess whether our product candidates are safe or effective in humans. Preclinical studies conducted in rats and non-human primates are not always indicative of clinical trial outcomes in humans.

We have not completed any clinical trials evaluating any of our product candidates or proposed delivery modes, including the use of lipid-based nanoparticles, or LNPs, that are customized for delivery to specific tissues.

There is a high failure rate for drugs and biologic products proceeding through preclinical studies and clinical trials. Any product candidates we develop may fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical studies and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of our product candidate development. Any such delays could materially and adversely affect our business, financial condition, results of operations and prospects.

***We may find it difficult to enroll and dose patients in our clinical trials, which could delay or prevent us from proceeding with clinical trials of our product candidates.***

Identifying, qualifying and enrolling patients to participate in clinical trials of our product candidates is critical to our success, and we may not be able to identify, recruit, enroll and dose a sufficient number of patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner. The timing of our clinical trials depends on our ability to recruit patients to participate as well as to subsequently dose these patients and complete required follow-up periods. In particular, because our clinical trial of MRT5005 and our SAD clinical trial of MRT5201 are focused on indications with relatively small patient populations, our ability to enroll eligible patients may be limited or may result in slower enrollment than we anticipate. Many CF clinical trial sites place importance on the review, ranking and sanctioning of CF patient advocacy groups. If CF patient advocacy groups do not timely sanction or highly rate our clinical trials, or prioritize trials of other sponsors over our trials, we may not be able to enroll sufficient patients to conduct our trials at their member sites, or it may take longer to conduct these trials.

In addition, we may experience enrollment delays related to increased or unforeseen regulatory, legal and logistical requirements at certain clinical trial sites. These delays could be caused by regulatory reviews by regulatory authorities and contractual discussions with individual clinical trial sites. Any delays in enrolling and/or dosing patients in our planned clinical trials could result in increased costs, delays in advancing our product candidates, delays in testing the effectiveness of our product candidates or termination of the clinical trials altogether.

Patient enrollment may be affected if our competitors have ongoing clinical trials for product candidates for the same indications as our product candidates, and patients who would otherwise be eligible for our clinical trials instead enroll in our competitors' clinical trials. Patient enrollment may also be affected by other factors, including:

- coordination between us, CROs and any future collaborators in our efforts to enroll and administer the clinical trial;

---

## Table of Contents

- size of the patient population and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- perceived risks and benefits of the product candidate under study;
- availability of competing commercially available therapies and other competing product candidates' clinical trials;
- time of year in which the trial is initiated or conducted;
- variations in the seasonal incidence of the target indication;
- severity of the disease under investigation;
- ability to obtain and maintain subject consent;
- ability to enroll and treat patients in a timely manner;
- risk that enrolled subjects will drop out before completion of the trial;
- proximity and availability of clinical trial sites for prospective patients;
- patient referral practices of physicians; and
- ability to monitor subjects adequately during and after treatment.

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

***We may not be successful in our efforts to identify or discover additional product candidates and may fail to capitalize on programs or product candidates for which there is a greater likelihood of commercial success.***

Our success depends upon our ability to identify, develop and commercialize product candidates based on our MRT platform. If we do not successfully develop and eventually commercialize products, we will not be able to generate product revenue, resulting in significant harm to our financial position and adverse effects to our share price. Research programs to identify new product candidates require substantial technical, financial and human resources. Although our product candidates are currently in preclinical or clinical development, we may fail to identify other potential product candidates for clinical development.

Additionally, because we have limited financial and managerial resources, we may forego or delay pursuit of opportunities for certain programs or product candidates or for indications that later prove to have greater commercial potential. For example, we currently intend to focus our capital resources primarily on the clinical development of MRT5005 and MRT5201. However, the development of MRT5005 and MRT5201 may ultimately prove to be unsuccessful or less successful than another product candidate in our pipeline that we might have chosen to pursue on a more aggressive basis with our capital resources. Our estimates regarding the potential market for our product candidates could be inaccurate, and our spending on current and future research and development programs may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights. Alternatively, we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a collaborative arrangement.

If any of these events occur, we may be forced to abandon or delay our development efforts with respect to a particular product candidate, or we may fail to develop a potentially successful product candidate, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

---

## [Table of Contents](#)

### ***We may fail to demonstrate safety and efficacy of our product candidates to the satisfaction of applicable regulatory authorities.***

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

- be delayed in obtaining marketing approval for our product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy, or REMS;
- be subject to the addition of labeling statements, such as contraindications or warnings, including a black box warning;
- be sued; or
- experience damage to our reputation.

### ***If serious adverse or undesirable side effects are identified during the development of our product candidates or proposed delivery modes, we may abandon or limit our development of such product candidates.***

If our product candidates or proposed delivery modes are associated with undesirable side effects or have unexpected characteristics, we may need to abandon their development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause side effects or raise other safety issues that delayed or prevented further development of the compound. Further, given the relatively small patient populations for which we are developing our product candidates, we expect to have to evaluate long-term exposure to establish the safety and tolerability of our product candidates in a chronic dose setting. The adverse effects from long-term exposure, as well as exposure in general, to our product candidates are unknown because they are a new class of therapeutics that have never been evaluated in a clinical trial. The risk of adverse or undesirable side effects therefore remains a significant concern, and we cannot assure you that these or other risks will not occur in any of our current or future clinical trials of MRT5005, MRT5201 or other product candidates that we may develop.

If we elect or are forced to suspend or terminate any clinical trial of our product candidates, the commercial prospects of such product candidate will be harmed, and our ability to generate product revenue from such product candidate will be delayed or eliminated. Any of these occurrences could materially harm our business, financial condition, results of operations and prospects.

### ***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 that these results may be difficult to analyze.***

During the regulatory review process, we will need to identify success criteria and endpoints such that the FDA, the EMA or other regulatory authorities will be able to determine the clinical efficacy and safety profile of any product candidates we may develop. Because our initial focus is 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 endpoints that we propose to provide clinically meaningful results. In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA determines that our success criteria is sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance.

---

## [Table of Contents](#)

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, the results may be 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. The EMA and other regulatory authorities may make similar comments with respect to these endpoints and data. Any product candidate 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.

***We may conduct clinical trials at sites outside the United States. The FDA may not accept data from trials conducted in such locations, and the conduct of trials outside the United States could subject us to additional delays and expense.***

We may conduct one or more of our clinical trials with one or more trial sites that are located outside the United States. Although the FDA may accept data from clinical trials conducted outside the United States, acceptance of these data is subject to certain conditions imposed by the FDA. For example, the clinical trial must be well designed and conducted and performed by qualified investigators in accordance with GCP. The FDA must be able to validate the data from the trial through an onsite inspection, if necessary. The trial population must also have a similar profile to the U.S. population, and the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful, except to the extent the disease being studied does not typically occur in the United States. In addition, while these clinical trials are subject to the applicable local laws, whether the FDA accepts the data will depend upon its determination that the trials also complied with all applicable U.S. laws and regulations. There can be no assurance that the FDA will accept data from trials conducted outside of the United States. If the FDA does not accept the data from any trial that we conduct outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and delay or permanently halt our development of MRT5005, MRT5201 or any future product candidates.

In addition, conducting clinical trials outside the United States could have a significant adverse impact on us. Risks inherent in conducting international clinical trials include:

- clinical practice patterns and standards of care that vary widely among countries;
- non-U.S. regulatory authority requirements that could restrict or limit our ability to conduct our clinical trials;
- administrative burdens of conducting clinical trials under multiple non-U.S. regulatory authority schema;
- foreign exchange fluctuations; and
- diminished protection of intellectual property in some countries.

***The manufacture of mRNA-based therapeutics is complex and manufacturers often encounter difficulties in production. If we or any of our third-party manufacturers encounter difficulties, our ability to provide product candidates for clinical trials or products, if approved, to patients could be delayed or halted.***

The manufacture of mRNA-based therapeutics is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. We and our third-party manufacturers must comply with current Good Manufacturing Practices, or cGMP, regulations and guidelines for the manufacturing of our product candidates used in preclinical studies and clinical trials and, if approved, marketed products. Manufacturers of biotechnology products often encounter difficulties in production, particularly in scaling up and validating initial production. Furthermore, if microbial, viral or other contaminations are discovered in our product candidates or in the manufacturing facilities where our product candidates are made, such manufacturing facilities may be closed for an extended period of time to investigate and remedy the contamination. Shortages of raw materials may also extend the period of time required to develop our product candidates.

We cannot assure you that any disruptions or other issues relating to the manufacture of any of our product candidates will not occur in the future. Any delay or interruption in the supply of clinical trial supplies could delay the completion of planned clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely. Any adverse developments affecting clinical or commercial manufacturing of our product candidates or products may result in shipment delays, inventory shortages, lot failures, product withdrawals or recalls or other interruptions in the supply of our product candidates or products. We may also have to take inventory write-offs and incur other charges and expenses for product candidates or products that fail to meet specifications, undertake costly remediation efforts or seek more costly manufacturing alternatives. Accordingly, failures or difficulties faced at any level of our supply chain could delay or impede the development and commercialization of any of our product candidates or products and could have an adverse effect on our business, prospects, financial condition and results of operations.

---

## [Table of Contents](#)

***If the market opportunities for our product candidates are smaller than we believe they are, even assuming approval of a product candidate, our business may suffer.***

Our product candidates are based on novel therapeutic approaches. As such, physicians, hospitals, third-party payors and patients may not accept our product candidates as treatment options, even if approved. While we believe there are commercial opportunities for our product candidates, we cannot be sure that is the case, particularly given the novelty of mRNA-based therapeutics.

Our projections of both the number of people affected by disease within our target indications, as well as the subset of these people who could benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including scientific literature, patient foundations and market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. Likewise, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or reach, which would adversely affect our results of operations and our business.

***We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.***

The biotechnology and pharmaceutical industries are highly competitive, characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We face and will continue to face competition from third parties that use mRNA, gene editing or gene therapy development platforms and from companies focused on more traditional therapeutic modalities, such as small molecules. The competition is likely to come from multiple sources, including large and specialty pharmaceutical and biotechnology companies, academic research institutions, government agencies and public and private research institutions.

Our competitors also include companies that are or will be developing other mRNA technology methods as well as small molecules, biologics and nucleic acid-based therapies for the same indications that we are targeting with our mRNA-based therapeutics.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even greater concentration of resources among a smaller number of competitors. Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products faster or earlier than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, new data from clinical-stage products continue to emerge. Technologies developed by our competitors may render our product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors' products. In addition, the availability of our competitors' products could limit the demand and the prices we are able to charge for any products that we may develop and commercialize.

If approved for the treatment of CF, MRT5005 would compete with Kalydeco, Orkambi and Symdeko, each of which is marketed by Vertex Pharmaceuticals Incorporated, or Vertex. Vertex also has several CFTR corrector compounds in clinical development, including VX-445, for which a new drug application has been filed with the FDA, and VX-659, as well as several others, each of which is currently in Phase 3 or Phase 2 clinical trials.

Our other potential competitors for CF include large pharmaceutical and biotechnology companies, specialty pharmaceutical and generic drug companies, academic institutions, government agencies and research institutions. Examples include AbbVie Inc., Corbus Pharmaceuticals, Inc., Eloxx Pharmaceuticals Ltd, Flatley Discovery Lab, LLC, Proteostasis Therapeutics, Inc., and Spyryx Biosciences, Inc.

Other companies developing products that modulate or affect CFTR function for the treatment of CF also include: CRISPR Therapeutics AG, Editas Medicine Inc. and Moderna, Inc.

There are currently no approved therapies that address the underlying cause of OTC deficiency. Competitors developing products that modulate or affect OTC function for the treatment of OTC deficiency include Ultragenyx Pharmaceuticals Inc., Kaleido Biosciences, Inc., Synlogic, Inc. and Arcturus Therapeutics Ltd. Several companies have developed and market treatments for OTC deficiency, including Horizon Pharma plc, Swedish Ophan Biovitrum AB and Bausch Health Companies Inc.

### **Risks Related to Dependence on Third Parties**

*We expect to depend on collaborations with third parties for the research, development, manufacture and commercialization of programs or product candidates. If these collaborations are not successful, our business could be adversely affected.*

As part of our strategy, we intend to seek to enter into collaborations with third parties for one or more of our programs or product candidates. For example, in June 2018, we entered into a collaboration and license agreement with Sanofi to develop mRNA vaccines for up to five infectious disease pathogens. Our likely collaborators for any other collaboration arrangements include large and mid-size pharmaceutical companies and biotechnology companies. If we enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that any future collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenue from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them.

Any collaborations we enter into, including our collaboration with Sanofi, may pose several risks, including the following:

- Collaborators may have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- Collaborators may not perform their obligations as expected;
- The clinical trials conducted as part of these collaborations may not be successful;
- Collaborators may not pursue development and/or commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- Collaborators may delay clinical trials, provide insufficient funding for clinical trials, 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;
- We may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates;
- Collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- Product candidates developed in collaboration with us may be viewed by any collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- A collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;
- Disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development of any programs or product candidates, may cause delays or termination of the research, development, manufacture or commercialization of such programs or product candidates, may lead to additional responsibilities for us with respect to such programs or product candidates or may result in litigation or arbitration, any of which would be time-consuming and expensive. Moreover, in certain circumstances, there could be a misalignment between the contractual obligations given to us by our collaborators and any upstream contractual obligations we may owe to our licensors or other third parties;

---

## [Table of Contents](#)

- Collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation. For example, Sanofi has the first right to enforce or defend certain of our intellectual property rights under our collaboration with respect to products in Licensed Fields, and although we may have the right to assume the enforcement and defense of such intellectual property rights if Sanofi does not, our ability to do so may be compromised by Sanofi's actions;
- Disputes may arise with respect to the ownership of intellectual property developed pursuant to our collaborations;
- Collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- Collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates. For example, Sanofi may terminate its collaboration with us for convenience after a specified notice period.

If our collaborations do not result in the successful development and commercialization of products, or if one of any future 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 our product candidates.

In addition, if any collaborator terminates its agreement with us, we may find it more difficult to attract new collaborators and our reputation among 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 also apply to the activities of our collaborators.

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

We face significant competition in attracting appropriate collaborators to advance the development of any product candidates for which we may seek a collaboration. 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, EMA or other regulatory authorities, 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, the terms of any existing collaboration agreements, and industry and market conditions generally. The collaborator may also have the opportunity to collaborate on other product candidates or technologies for similar indications and will have to evaluate whether such a collaboration could be more attractive than one with us.

Collaborations are complex and time-consuming to negotiate, document and execute. In addition, consolidation among large pharmaceutical companies has reduced the number of potential future collaborators.

Under the Shire Agreement, prior to the first dosing of a patient with a CFTR MRT Product in a Phase 3 clinical trial, Shire has a 90-day right of first negotiation before we may grant rights or sell assets relating to our CFTR MRT Products to a third party. Shire may exercise the right of first negotiation for a period of 30 days following Shire's receipt of written notice from us notifying Shire of the offer from a third party to acquire, license or commercialize grant rights or sell assets relating to our CF program.

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 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, which could have an adverse effect on our business, prospects, financial condition and results of operations.

***We expect to rely on third parties to conduct our clinical trials and some aspects of our research and preclinical studies, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research or testing.***

We currently rely and expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct our clinical trials. In addition, we currently rely and expect to continue to rely on third parties to conduct some aspects of our research and preclinical studies. Any of these third parties may terminate their engagements with us, some in the event of an uncured material breach and some at any time for convenience. If any of our relationships with these third parties terminate, we may not be able to enter into alternative arrangements on commercially reasonable terms, if at all. Switching or including additional third parties involves increased cost and requires management's time and focus. In addition, there is a natural transition period when a new third party commences work. As a result, delays may occur in our product development activities. Although we seek to carefully manage our relationships with our third parties, we could encounter similar challenges or delays in the future and these challenges or delays could have a material adverse impact on our business, financial condition and prospects.

Our reliance on third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards. We and these third parties are required to comply with GCP, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable regulatory authorities, for all of our products in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these third parties fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, the EMA or comparable regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with products produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a U.S. government-sponsored database, [clinicaltrials.gov](http://clinicaltrials.gov), within certain timeframes. Similar requirements are applicable outside the United States. Failure to comply can result in fines, adverse publicity and civil and criminal sanctions.

Furthermore, third parties on whom we rely may also have relationships with other entities, some of which may be our competitors. In addition, these third parties are not our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our ongoing clinical, non-clinical and preclinical programs. If these third parties do not successfully satisfy 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, our preclinical studies or clinical trials may be extended, delayed or terminated, and we may not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our products. As a result, our results of operations and the commercial prospects for our products would be harmed, our costs could increase and our ability to generate revenue could be impaired.

***Our reliance on third parties to manufacture our product candidates and any future products increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.***

We do not own or operate manufacturing facilities for the production of clinical or commercial supplies of the product candidates that we are developing or evaluating in our research program. We have limited personnel with experience in drug manufacturing and lack the resources and capabilities to manufacture any of our product candidates on a clinical or commercial scale. We currently rely on third parties for supply of our product candidates, and we outsource to third parties all manufacturing of our product candidates in preparation for our clinical trials.

In order to conduct clinical trials of our product candidates, we will need to have them manufactured in potentially large quantities. Our third-party manufacturers may be unable to meet this increased demand in a timely or cost-effective manner, or at all. In addition, quality issues may arise during scale-up activities and at any other time. For example, ongoing data on the stability of our products may shorten the expiry of our products and lead to clinical trial material supply shortages, and potentially clinical trial delays. If these third-party manufacturers are unable to successfully scale up the manufacture of our product candidates in sufficient quality and quantity, the development, testing and clinical trials of that product candidate may be delayed or infeasible, and regulatory approval or commercial launch of that product candidate may be delayed or not obtained, which could significantly harm our business.

---

## [Table of Contents](#)

Our use of new third-party manufacturers increases the risk of delays in production or insufficient supplies of our product candidates as we transfer our manufacturing technology to these manufacturers and as they gain experience manufacturing our product candidates.

Even after a third-party manufacturer has gained significant experience in manufacturing our product candidates or even if we believe we have succeeded in optimizing the manufacturing process, there can be no assurance that such manufacturer will produce sufficient quantities of our product candidates in a timely manner or continuously over time, or at all.

We do not currently have any agreements with third-party manufacturers for the long-term commercial supply of any of our product candidates. In the future, we may be unable to enter into such agreements with third-party manufacturers for commercial supplies of our product candidates, or may be unable to do so on acceptable terms. Even if we are able to establish and maintain arrangements with third-party manufacturers, reliance on third-party manufacturers entails risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- 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.

Third-party manufacturers may not be able to comply with cGMP requirements or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable requirements could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and/or criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

Our product candidates and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP requirements, particularly for the development of mRNA-based therapeutics, and that might be capable of manufacturing for us.

If the third parties that we engage to supply any materials or manufacture product for our preclinical tests and clinical trials should cease to do so for any reason, we likely would experience delays in advancing these tests and trials while we identify and qualify replacement suppliers or manufacturers, and we may be unable to obtain replacement supplies on terms that are favorable to us. For example, we rely on one third-party supplier of the handheld nebulizer that patients in our clinical trials use to administer MRT5005. The failure of our supplier to provide sufficient quantities, acceptable quality and timely delivery of the nebulizer at an acceptable price, or an interruption in the delivery of goods from such supplier, could delay or otherwise adversely affect our clinical trials of MRT5005, and harm our business and prospects. The use of an alternative manufacturer of the nebulizer could involve significant delays and other costs and regulatory challenges, and may not be available to us on reasonable terms, if at all. In addition, if we are not able to obtain adequate supplies of our product candidates or the substances used to manufacture them, it will be more difficult for us to develop our product candidates and compete effectively.

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

### **Risks Related to the Commercialization of our Product Candidates**

***If we are unable to establish sales, medical affairs and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any product revenue.***

We do not currently have a sales and marketing organization and have never commercialized a product. To successfully commercialize any products that may result from our development programs, we will need to develop these capabilities, either on our own or with others. The establishment and development of our own commercial and medical science liaison teams or the engagement of a contract sales force will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We have entered into a collaboration with Sanofi and may also seek to enter into future collaborations with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If our collaborators do not commit sufficient resources to commercialize our products, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many well-funded and profitable pharmaceutical and biotechnology companies that currently have extensive and experienced medical affairs, marketing and sales operations to recruit,

---

## [Table of Contents](#)

hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. Without an internal team or the support of a third party to perform marketing, sales and medical affairs functions, we may be unable to compete successfully against these more established companies.

Our efforts to educate the medical community and third-party payors about the benefits of our product candidates may require significant resources and may never be successful. If any of our product candidates are approved but fail to achieve market acceptance among physicians, patients, hospitals or third-party payors, we will not be able to generate significant revenue from such product, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

***The hospital formulary approval and insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate hospital formulary approval and/or insurance coverage and reimbursement for our products, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.***

We expect that hospital formulary approval and insurance coverage and reimbursement by government and other third-party payors of our products, if approved, will be essential for most patients to be able to access these treatments. Accordingly, sales of our product candidates, if approved, will depend substantially on the extent to which the costs of our product candidates will be paid by hospitals or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Hospital formulary approval and insurance coverage and reimbursement by other third-party payors may depend upon several factors, including the third-party payor's determination that use of a product is:

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

Obtaining hospital formulary approval and insurance coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that will require us to provide to the hospitals and payors supporting scientific, clinical and cost-effectiveness data. We may not be able to provide data sufficient to gain acceptance with respect to hospital formulary approval and insurance coverage and reimbursement. If hospital formulary approval, insurance coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our product candidates.

There is significant uncertainty related to hospital formulary approval and insurance coverage and reimbursement of newly approved products. In the United States, third-party payors, including government payors such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. It is difficult to predict what third-party payors will decide with respect to the insurance coverage and reimbursement for our product candidates.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations, and increasing emphasis on cost-containment initiatives in the European Union, Canada and other countries may put pricing pressure on us. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries may use different methods to keep the cost of medical products artificially low. Foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable product revenue.

Moreover, hospitals and government and other third-party payors in the United States and abroad have increasingly taken measures to cap or reduce health care costs. For example, governmental and other third-party payors may attempt to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward reducing hospital costs, managed health care, the increasing influence of health maintenance organizations and additional legislative changes.

---

## [Table of Contents](#)

***The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, hospitals, third-party payors and others in the medical community.***

Even with the requisite approvals from the FDA in the United States, EMA in the European Union and other regulatory authorities internationally, the commercial success of our product candidates, if approved, will significantly depend on the acceptance of physicians, hospitals and health care payors of our product candidates as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, hospitals, health care payors and others in the medical community. If these commercialized products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of our product candidates over other treatments;
- the cost-effectiveness of treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA, the EMA or other regulatory body;
- the willingness of physicians to prescribe new therapies over the existing standard of care and future 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, including any black box warning;
- relative convenience and ease of administration;
- our ability to educate the medical community and third-party payors about the benefit of our product candidates;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- any restrictions on the use of our products together with other medications;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party payor insurance coverage and adequate reimbursement.

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 we begin to commercialize the product.

***If we obtain approval to commercialize our product candidates outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.***

We expect that we will be subject to additional risks in commercializing our product candidates outside the United States, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;

---

## [Table of Contents](#)

- economic weakness, including inflation, or political instability in foreign economies and markets;
- different pricing and reimbursement regimes;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters, including earthquakes, typhoons, floods and fires.

### **Risks Related to Our Business Operations**

#### ***Our future success depends on our ability to retain key employees, consultants and advisors and to attract, retain and motivate qualified personnel.***

We are highly dependent on members of our executive team. The loss of the services of any of them may adversely impact the achievement of our objectives. Any of our executive officers could leave our employment at any time, as all of our employees are “at-will” employees.

Recruiting and retaining qualified employees, consultants and advisors for our business, including scientific and technical personnel, is also critical to our success. Competition for skilled personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for skilled individuals. In addition, failure to succeed in preclinical studies, clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of certain executives, key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives and have a material adverse effect on our business, financial condition, results of operations and prospects.

#### ***If we are unable to manage expected growth in the scale and complexity of our operations, our performance may suffer.***

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities and, in the longer term, build a commercial infrastructure to support commercialization of any of our product candidates that are approved for sale. Future growth would impose significant added responsibilities on members of management. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

#### ***Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any product candidates that we may develop.***

We face an inherent risk of product liability exposure related to the testing of our product candidates in clinical trials and may face an even greater risk if we commercialize any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates that we may develop;
- loss of revenue;
- substantial monetary awards to trial participants or patients;

---

## [Table of Contents](#)

- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to commercialize any product candidates that we may develop; and
- injury to our reputation and significant negative media attention.

Our insurance coverage may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any product candidate. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

***Our internal computer systems, or those of any collaborators, 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 any collaborators, 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 material 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 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. 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. In addition, we may not have adequate insurance coverage to provide compensation for any losses associated with such events.

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, including personal information of our employees. In addition, outside parties may attempt to penetrate our systems or those of our vendors or fraudulently induce our employees or employees of our vendors to disclose sensitive information to gain access to our data. Like other companies, we may experience threats to our data and systems, including malicious codes and viruses, and other cyber-attacks. The number and complexity of these threats continue to increase over time. If a material breach of our security or that of our vendors occurs, the market perception of the effectiveness of our security measures could be harmed, we could lose business 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. 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 more sophisticated. Moreover, despite our efforts, the possibility of these events occurring cannot be eliminated entirely.

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

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include failures to:

- comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions;
- provide accurate information to the FDA, the EMA and other regulatory authorities;
- comply with health care fraud and abuse laws and regulations in the United States and abroad;
- comply with the U.S. Foreign Corrupt Practices Act, or FCPA, or other anti-corruption laws and regulations;
- comply with U.S. federal securities laws relating to trading in our common stock;
- report financial information or data accurately; or
- disclose unauthorized activities to us.

---

## [Table of Contents](#)

In particular, sales, marketing and business arrangements in the health care industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations regulate a wide range of pricing, discounting, marketing and promotional practices, as well as sales and customer incentive programs and other business arrangements. Other forms of misconduct could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA, EMA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct and expect to implement other internal controls applicable to all of our employees, consultants and contractors, but it is not always possible to identify and deter third-party misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us and we are not successful in defending ourselves or asserting our rights, we may be subject to civil, criminal and/or administrative penalties, damages, fines, disgorgement, individual imprisonment, exclusion from participation in government health care programs, contractual damages, reputational harm, administrative burdens, diminished profits and future earnings, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the curtailment or restructuring of our operations, any of which could have a significant impact on our business, financial condition, results of operations and prospects.

### **Risks Related to Our Intellectual Property**

***If we are unable to obtain and maintain patent protection for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad or robust, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our products and technology may be adversely affected.***

Our success depends, in large part, on our ability to obtain and maintain patent protection in the United States and other countries with respect to our product candidates and technology. We and our licensors have sought, and intend to seek, to protect our proprietary position by filing patent applications in the United States and abroad related to our product candidates and technology that are important to our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we or our licensors were the first to file a patent application relating to any particular aspect of a product candidate. Furthermore, if third parties have filed such patent applications on inventions claimed in our patent or patent application on or before March 15, 2013, an interference proceeding in the United States can be initiated by such third party 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 applications after March 15, 2013, a derivation proceeding in the United States can be initiated by such third parties to determine whether our invention was derived from theirs.

The patent prosecution process is expensive, time-consuming and complex, and we may not be able to file, prosecute, maintain, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the United States Patent and Trademark Office, or USPTO, and various government patent agencies outside of the United States over the lifetime of our licensed patents and/or applications and any patent rights we own or may own in the future. We rely, in part, on our outside counsel or our licensing partners to pay these fees due to the USPTO and to non-U.S. patent agencies. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance 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. In such an event, potential competitors might be able to enter the market and this circumstance could have a material adverse effect on our business.

---

## [Table of Contents](#)

Filing, prosecuting and enforcing patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from infringing our patents in all countries outside the United States, or from selling or importing products that infringe our patents in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Even if the patent applications we license or own do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, 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 product candidates. 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 intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

### ***Our product candidates may face competition from biosimilars approved through an abbreviated regulatory pathway.***

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

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

### ***Changes in patent law could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.***

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity, and obtaining and enforcing biopharmaceutical patents is costly, time consuming and inherently uncertain. The U.S. Supreme Court has ruled on several patent cases in recent years, and these decisions have narrowed the scope of patent protection available in certain circumstances or weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our and our licensors' 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 future decisions by the U.S. Congress, the federal courts and the USPTO, as well as similar bodies in foreign jurisdictions, the laws and regulations governing patents could change in unpredictable ways that may weaken our and our licensors' ability to obtain new patents or to enforce existing patents and patents we and our licensors or any collaborators may obtain in the future.

Patent reform legislation enacted in the United States in 2011 could increase the uncertainties and costs surrounding the prosecution of our and our licensors' patent applications and the enforcement or defense of our or our licensors' issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act

---

## [Table of Contents](#)

includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art, may affect patent litigation and switch the U.S. patent system from a “first to invent” system to a “first inventor to file” system. The USPTO has developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular the first inventor to file provisions, became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our or our licensors’ patent applications and the enforcement or defense of our or our licensors’ issued patents, all of which could have a material adverse effect on our business and financial condition.

***Our rights to develop and commercialize our product candidates are subject, in part, to the terms and conditions of licenses granted to us by others, and, if we fail to comply with our obligations under these arrangements, we could lose such intellectual property rights or owe damages to the licensor of such intellectual property.***

We are a party to several intellectual property license agreements, including agreements with the Massachusetts Institute of Technology, or MIT, that are important to our business, and may need to obtain additional licenses from others to advance our research or allow commercialization of our product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and product candidates in the future. It is possible that we may be unable to obtain additional licenses at a reasonable cost or on reasonable terms, if at all. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses. In that event, we may be required to expend significant time and resources to redesign our product candidates or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly.

Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, development and commercialization timelines, milestone payments, royalties and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

For example, our license agreement with MIT imposes specified diligence, annual payment, milestone payment, royalty and other obligations on us. If we fail to comply with our obligations under the license agreement, MIT may have the right to terminate the license agreement, in which event we might not be able to market, and may be required to transfer to MIT our rights in, any product that is covered by the MIT agreement, including MRT5201 and products that may be developed under our collaboration with Sanofi. Termination of the license agreement may also result in our having to negotiate a new or reinstated license with less favorable terms, which would have a material adverse impact on our business.

In our existing license agreements, and we expect in future agreements, patent prosecution of our licensed technology is in certain cases controlled solely by the licensor, and we are in certain cases required to reimburse the licensor for their costs of patent prosecution. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products covered by the intellectual property. Further, in each of our license agreements, we are responsible for bringing any actions against any third party for infringing the patents we have licensed. Certain of our license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing products and minimum yearly diligence obligations in developing and commercializing the product. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

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

---

## [Table of Contents](#)

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

Competitors may infringe our patents or the patents of our licensing partners, or we may be required to defend against claims of infringement. Countering infringement or unauthorized use claims or defending against claims of infringement can be expensive and time-consuming. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

In addition, many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. 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 own, develop or license.

***Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court. We may not be able to protect our trade secrets in court.***

If we or one of our licensing partners initiates legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, written description or non-enablement. In addition, patent validity challenges may, under certain circumstances, be based upon non-statutory obviousness-type double patenting, which, if successful, could result in a finding that the claims are invalid for obviousness-type double patenting or the loss of patent term, including a patent term adjustment granted by the USPTO, if a terminal disclaimer is filed to obviate a finding of obviousness-type double patenting. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions. For example, as of July 29, 2019, three of our patents issued in Europe are under opposition, including one with claims of similar scope as U.S. Patent 10,143,758. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art of which the patent examiner and we or our licensing partners were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates. Such a loss of patent protection could have a material adverse impact on our business.

---

## [Table of Contents](#)

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect, and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

***Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.***

Our commercial success depends upon our ability and the ability of any collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current manufacturing methods, product candidates or future methods or products, resulting in either an injunction prohibiting our manufacture or sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including interference proceedings, post grant review and *inter partes* review before the USPTO. The risks of being involved in such litigation and proceedings may also increase as our product candidates approach commercialization and as we gain greater visibility as a public company. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could materially and adversely affect our ability to commercialize any of our product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent.

If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue developing, manufacturing and marketing our product candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects.

***Others may claim an ownership interest in our intellectual property and our product candidates, which could expose us to litigation and have a significant adverse effect on our prospects.***

While we are presently unaware of any claims or material assertions by third parties with respect to our patents or other intellectual property, we cannot guarantee that a third party will not assert a claim or an interest in any of such patents or intellectual property. For example, a third party may claim an ownership interest in one or more of our, or our licensors', patents or other proprietary or intellectual property rights. A third party could bring legal actions against us and seek monetary damages or enjoin clinical testing, manufacturing or marketing of the affected product candidate or product. If we become involved in any litigation, it could consume a substantial portion of our resources and cause a significant diversion of effort by our technical and management personnel. If any such action is successful, in addition to any potential liability for damages, we could be required to obtain a license to continue to manufacture or market the affected product candidate or product, in which case we could be required to pay substantial royalties or grant cross-licenses to patents. We cannot, however, assure you that any such license would be available on acceptable terms, if at all. Ultimately, we could be prevented from commercializing a product, or forced to cease some aspect of our business.

---

## [Table of Contents](#)

operations as a result of claims of patent infringement or violation of other intellectual property rights. Further, the outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance, including the demeanor and credibility of witnesses and the identity of any adverse party. This is especially true in intellectual property cases, which may turn on the testimony of experts as to technical facts upon which experts may reasonably disagree. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations or prospects.

***If we are unable to protect the confidentiality of our proprietary information, the value of our technology and products could be adversely affected.***

Trade secrets and know-how can be difficult to protect. To maintain the confidentiality of trade secrets and proprietary information, we enter into confidentiality agreements with our employees, consultants, collaborators and others upon the commencement of their relationships with us. These agreements require that all confidential information developed by the individual or made known to the individual by us during the course of the individual's relationship with us be kept confidential and not disclosed to third parties. Our agreements with employees and our personnel policies also provide that any inventions conceived by the individual in the course of rendering services to us shall be our exclusive property. However, we may not obtain these agreements in all circumstances, and individuals with whom we have these agreements may not comply with their terms. Thus, despite such agreement, there can be no assurance that such inventions will not be assigned to third parties. In the event of unauthorized use or disclosure of our trade secrets or proprietary information, these agreements, even if obtained, may not provide meaningful protection, particularly for our trade secrets or other confidential information. To the extent that our employees, consultants or contractors use technology or know-how owned by third parties in their work for us, disputes may arise between us and those third parties as to the rights in related inventions. To the extent that an individual who is not obligated to assign rights in intellectual property to us is rightfully an inventor of intellectual property, we may need to obtain an assignment or a license to that intellectual property from that individual, or a third party or from that individual's assignee. Such assignment or license may not be available on commercially reasonable terms or at all.

Adequate remedies may not exist in the event of unauthorized use or disclosure of our proprietary information. The disclosure of our trade secrets would impair our competitive position and may materially harm our business, financial condition and results of operations. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to maintain trade secret protection could adversely affect our competitive business position. In addition, others may independently discover or develop our trade secrets and proprietary information, and the existence of our own trade secrets affords no protection against such independent discovery. For example, a public presentation in the scientific or popular press on the properties of our product candidates could motivate a third party, despite any perceived difficulty, to assemble a team of scientists having backgrounds similar to those of our employees to attempt to independently reverse engineer or otherwise duplicate our antibody technologies to replicate our success.

***We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.***

Many of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals, or we, have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer, or that patents and applications we have filed to protect inventions of these employees, even those related to one or more of our product candidates, are rightfully owned by their former or current employer. 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.

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

***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.***

Any registered trademarks or trade names may be challenged, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or

---

## [Table of Contents](#)

trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

### ***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 our product candidates but that are not covered by the claims of the patents that we own or license or may own in the future;
- we, or any partners or collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- we, or any partners or collaborators, 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 licensed patent applications or those that we may own 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 have an adverse effect on our business; and
- we may choose not to file a patent for 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 significantly harm our business, financial condition, results of operations and prospects.

### **Risks Related to Regulatory Approval and Other Legal Compliance Matters**

***The regulatory approval process of the FDA is lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.***

The time required to obtain approval by the FDA is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate and it is possible that none of our existing product candidates, or any product candidates we may seek to develop in the future, will ever obtain regulatory approval.

---

## [Table of Contents](#)

Our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA that a product candidate is safe, pure and potent or effective for its proposed indication;
- results of clinical trials may not meet the level of statistical significance required by the FDA for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA may disagree with our interpretation of data from preclinical studies or clinical trials;
- data collected from clinical trials of our product candidates may not be sufficient to support the submission of a BLA to the FDA or other submission or to obtain regulatory approval in the United States;
- the FDA may find deficiencies with or fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market any of our product candidates, which would significantly harm our business, results of operations and prospects. The FDA has substantial discretion in the approval process, and determining when or whether regulatory approval will be obtained for any of our product candidates. Even if we believe the data collected from clinical trials of our product candidates are promising, such data may not be sufficient to support approval by the FDA.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

***Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us or any future collaborators from obtaining approvals for the commercialization of some or all of our product candidates. As a result, we cannot predict when or if, and in which territories, we, or any future collaborators, will obtain marketing approval to commercialize a product candidate.***

The research, testing, manufacturing, labeling, approval, selling, marketing, promotion and distribution of drug products are subject to extensive regulation by the FDA, EMA and other regulatory authorities, and regulations may differ from country to country. We, and any future collaborators, are not permitted to market our product candidates in the United States or in other countries until we, or they, receive approval of a BLA from the FDA, approval of a marketing authorization application, or MAA, from the EMA, or marketing approval from other applicable regulatory authorities. Our product candidates are in various stages of development and are subject to the risks of failure inherent in drug development. We have not submitted an application for or received marketing approval for any of our product candidates in the United States, Europe or in any other jurisdiction. We have not yet been successful at conducting and managing the clinical trials necessary to obtain marketing approvals, including FDA approval of a BLA and EMA approval of an MAA.

The process of obtaining marketing approvals, both in the United States and abroad, is lengthy, expensive and uncertain. It may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved.

In addition, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical studies and clinical trials could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or any future collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

---

## [Table of Contents](#)

Any delay in obtaining or failure to obtain required approvals could materially adversely affect our ability or that of any future collaborators to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price.

***Failure to obtain marketing approval in foreign jurisdictions would prevent our product candidates from being marketed abroad and may limit our ability to generate revenue from product sales.***

In order to market and sell our products in the European Union and many other jurisdictions, we, and any future 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 marketing approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. We, and any future collaborators, may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA.

In many countries outside the United States, a product candidate must also be approved for reimbursement before it can be sold in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. Obtaining non-U.S. regulatory approvals and compliance with non-U.S. regulatory requirements could result in significant delays, difficulties and costs for us and any future collaborators and could delay or prevent the introduction of our product candidates in certain countries. In addition, if we or any future collaborators fail to obtain the non-U.S. approvals required to market our product candidates outside the United States or if we or any future collaborators fail to comply with applicable non-U.S. regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects may be adversely affected.

Additionally, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. On March 29, 2017, the country formally notified the European Union of its intention to withdraw pursuant to Article 50 of the Lisbon Treaty. Since a significant proportion of the regulatory framework in the United Kingdom is derived from European Union directives and regulations, Brexit could materially impact the regulatory regime with respect to the approval of our product candidates in the United Kingdom or the European Union. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for our product candidates, which could significantly and materially harm our business.

The United Kingdom had a period of a maximum of two years from the date of its formal notification to negotiate the terms of its withdrawal from, and future relationship with, the European Union. If no formal withdrawal agreement can be reached between the United Kingdom and the European Union, then it is expected that the United Kingdom's membership in the European Union would automatically terminate on the deadline, which has been extended to October 31, 2019 to allow the parties to negotiate a withdrawal agreement. Such negotiations have proven to be extremely difficult to date. Discussions between the United Kingdom and the European Union will continue to focus on finalizing withdrawal issues and transition agreements. However, limited progress to date in these negotiations and ongoing uncertainty within the UK Government and Parliament sustains the possibility of the United Kingdom leaving the European Union on March 29, 2019 without a withdrawal agreement and associated transition period in place, which is likely to cause significant market and economic disruption.

***We, or any future collaborators, may not be able to obtain and maintain orphan drug exclusivity for our product candidates in the United States and Europe.***

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs and biologics for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. In November 2015, the FDA granted orphan drug designation to MRT5005 for the treatment of CF, and in March and June 2018, MRT5201 was granted orphan drug designation for the treatment of OTC deficiency in the U.S. and the EU, respectively. We may seek orphan drug designations for MRT5005 and MRT5201 for other indications or for other of our product candidates. There can be no assurances that we will be able to obtain such designations.

---

## [Table of Contents](#)

Even if we, or any future collaborators, obtain orphan drug designation for a product candidate as we have obtained for MRT5005 for the treatment of CF and for MRT5201 for the treatment of OTC deficiency, we, or they, may not be able to obtain or maintain orphan drug exclusivity for that product candidate. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the EMA or the FDA from approving another marketing application for the same product for that time period. The applicable period is seven years in the United States and 10 years in Europe. The European exclusivity period can be reduced to six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition. Moreover, even after an orphan drug is approved, the FDA can subsequently approve a different product for the same condition if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

On August 3, 2017, Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's pre-existing 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 new legislation 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. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. 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.

***We may seek fast track designation by the FDA for one or more of our product candidates, but we might not receive such designation, and even if we do, such designation may not actually lead to a faster development or regulatory review or approval process.***

If a product is intended for the treatment of a serious or life-threatening condition and the product demonstrates the potential to address unmet needs for this condition, the treatment sponsor may apply for FDA fast track designation. If we seek fast track designation for a product candidate, we may not receive it from the FDA. However, even if we receive fast track designation, fast track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. We may not experience a faster development, regulatory review or approval process with fast track designation compared to conventional FDA procedures. Additionally, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

***A breakthrough therapy designation by the FDA for a product candidate may not lead to a faster development or regulatory review or approval process, and it would not increase the likelihood that the product candidate will receive marketing approval.***

We may seek a breakthrough therapy designation for one or more product candidates. A breakthrough therapy is defined as a product candidate that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the product candidate may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Product candidates designated as breakthrough therapies by the FDA are also eligible for priority review if supported by clinical data at the time of the submission of the BLA.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that 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 product candidates considered for approval under conventional FDA procedures and it would 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 candidate no longer meets the conditions for qualification or it may decide that the time period for FDA review or approval will not be shortened.

---

## [Table of Contents](#)

***Even if we, or any future collaborators, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our products may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.***

Once marketing approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and any future collaborators, must therefore comply with requirements concerning advertising and promotion for any of our product candidates for which we or they obtain marketing approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we, and any future collaborators, will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our third-party manufacturers, any future collaborators and their third-party manufacturers could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMPs.

Accordingly, assuming we, or any future collaborators, receive marketing approval for one or more of our product candidates, we, and any future collaborators, and our respective third-party 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 any future collaborators, are not able to comply with post-approval regulatory requirements, we, and any future collaborators, could have the marketing approvals for our products withdrawn by regulatory authorities and our, or any future 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 operating results and financial condition.

***Any of our product candidates for which we, or any future collaborators, obtain marketing approval in the future could be subject to post-marketing restrictions or withdrawal from the market and we, or any future collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.***

Any of our product candidates for which we, or any future collaborators, obtain marketing approval in the future, as well as the manufacturing processes, post-approval studies and measures, labeling, advertising and promotional activities for such product, among other things, will be subject to continual requirements of and review by the FDA, EMA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a REMS.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we, or any future collaborators, do not market any of our product candidates for which we, or they, receive marketing approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing. Violation of the Federal Food, Drug, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws.

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;

---

## Table of Contents

- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- restrictions on coverage by third-party payors;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals, including license revocation;
- refusal to permit the import or export of products;
- product seizure; and
- injunctions or the imposition of civil or criminal penalties.

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

Health care providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any drugs for which we obtain marketing approval. Our future arrangements with third-party payors, health care providers and physicians may expose us to broadly applicable fraud and abuse and other health care laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any drugs for which we obtain marketing approval. These include the following:

- *Anti-Kickback Statute*—the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing any remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation or arranging of, any good, facility, item or service, for which payment may be made, in whole or in part, by a federal health care program, such as Medicare and Medicaid;
- *False Claims Act*—the federal civil and criminal false claims laws impose criminal and civil penalties, including, in some cases, through civil whistleblower or qui tam actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, false or fraudulent claims for payment by a federal health care program or knowingly making a false statement or record material to payment of a false claim or knowingly avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties;
- *HIPAA*—the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit, among other things, executing a scheme to defraud any health care benefit program or making false statements relating to health care matters, and apply regardless of the payor (e.g., public or private);
- *HIPAA and HITECH*—HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, which impose obligations on HIPAA covered entities and their business associates, including mandatory contractual terms and required implementation of administrative, physical and technical safeguards to maintain the privacy and security of individually identifiable health information;
- *Transparency Requirements*—federal transparency laws, including the federal Physician Payments Sunshine Act, require applicable manufacturers of covered drugs to annually report payments and other transfers of value to physicians and teaching hospitals and ownership or investment interests held by physicians and their family members; and

---

## [Table of Contents](#)

- *Analogous State and Foreign Laws*—analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, which may be broader than similar federal laws, can apply to claims involving health care items or services regardless of payor, and are enforced by many different federal and state agencies as well as through private actions.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and require drug manufacturers to report information related to payments and other transfers of value to physicians and other health care providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not pre-empted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable health care laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other health care laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and/or administrative penalties, damages, fines, individual imprisonment, disgorgement, exclusion of drugs from government funded health care programs, such as Medicare and Medicaid, contractual damages, reputational harm, administrative burdens, diminished profits and future earnings, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the curtailment or restructuring of our operations. If any of the physicians or other health care providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded health care programs.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the European Union. The provision of benefits or advantages to physicians is governed by the national anti-bribery laws of European Union Member States, such as the U.K. Bribery Act 2010, or the Bribery Act. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

The collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the EU, including personal health data, is subject to the EU General Data Protection Regulation 2016/679, or GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EU, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. Compliance with the GDPR will be 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 European activities.

---

## [Table of Contents](#)

### ***Current and future legislation may increase the difficulty and cost for us and any future collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.***

In the United States and some foreign jurisdictions, there have been and continue to be a number of legislative and regulatory changes and proposed changes regarding the health care 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, or the ability of any future collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that current laws, as well as other health care reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any future collaborators, may receive for any approved products.

In March 2010, President Obama signed into law the ACA. Among the provisions of the ACA of importance to our business, including, without limitation, our ability to commercialize and the prices we may obtain for any of our product candidates, are the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic products;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of federal health care fraud and abuse laws, including the civil False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report certain financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These changes include the Budget Control Act of 2011, which, among other things, led to aggregate reductions to Medicare payments to providers of 2% per fiscal year starting in 2013 and that, due to subsequent legislative amendments to the statute, will stay in effect through 2025 unless additional congressional action is taken, and the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws and any new health care reform measures may result in additional reductions in Medicare and other health care 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. Further, there have been several recent U.S. congressional inquiries and proposed state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products.

Since enactment of the ACA, there have been numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, which was signed by the President on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. According to the Congressional Budget Office, the repeal of the individual mandate will cause 13 million fewer Americans to be insured in 2027 and premiums in insurance markets may rise. Further, each chamber of Congress has put forth multiple bills designed to repeal or repeal and replace portions of the ACA. Although none of these measures has been enacted by Congress to date, Congress may consider other legislation to repeal and replace elements of the ACA. Congress will likely consider other legislation to replace elements of the ACA during the next Congressional session. It is possible that repeal and replacement initiatives, if enacted into law, could ultimately result in fewer individuals having health

---

## [Table of Contents](#)

insurance coverage or in individuals having insurance coverage with less generous benefits. While the timing and scope of any potential future legislation to repeal and replace ACA provisions is highly uncertain in many respects, it is also possible that some of the ACA provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with ACA coverage expansion provision.

We expect that these health care reforms, as well as other health care reform measures that may be adopted in the future, may result in additional reductions in Medicare, Medicaid and other health care funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or administered. Any reduction in reimbursement from Medicare, Medicaid or other government programs may result in a similar reduction in payments from private payors.

The Trump Administration has also taken executive actions to undermine or delay implementation of the ACA. Since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. One Executive Order directs federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. The second Executive Order terminates the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the Administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Further, on June 14, 2018, the U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than \$12 billion in ACA risk corridor payments to third-party payors who argued such payments were owed to them. The effects of this gap in reimbursement on third-party payors, the viability of the ACA marketplace, providers, and potentially our business, are not yet known.

The cost of prescription pharmaceuticals has also been the subject of considerable discussion in the United States, and members of Congress and the Administration have stated that they will address such costs through new legislative and administrative measures. To date, there have been several recent U.S. Congressional inquiries and proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, the Trump Administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. While any proposed measures will require authorization through additional legislation to become effective, Congress and the Trump Administration have each indicated that they will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and proposed and enacted state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, Congress and the Trump Administration have each indicated that they will continue to seek new legislative and/or administrative measures to control drug costs. For example, on May 11, 2018, the Administration issued a plan to lower drug prices. Under this blueprint for action, the Administration indicated that the Department of Health and Human Services will: take steps to end the gaming of regulatory and patent processes by drug makers to unfairly protect monopolies; advance biosimilars and generics to boost price competition; evaluate the inclusion of prices in drug makers' ads to enhance price competition; speed access to and lower the cost of new drugs by clarifying policies for sharing information between insurers and drug makers; avoid excessive pricing by relying more on value-based pricing by expanding outcome-based payments in Medicare and Medicaid; work to give Part D plan sponsors more negotiation power with drug makers; examine which Medicare Part B drugs could be negotiated for a lower price by Part D plans, and improving the design of the Part B Competitive Acquisition Program; update Medicare's drug-pricing dashboard to increase transparency; prohibit Part D contracts that include "gag rules" that prevent pharmacists from informing patients when they could pay less out-of-pocket by not using insurance; and require that Part D plan members be provided with an annual statement of plan payments, out-of-pocket spending, and drug price increases.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage

---

## [Table of Contents](#)

importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

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

***Governments outside of the United States tend to impose strict price controls, which may adversely affect our revenues from the sales of drugs, if any.***

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a drug. To obtain reimbursement or pricing approval in some countries, we, or our future collaborators, may be required to conduct a clinical trial that compares the cost-effectiveness of our drug to other available therapies. If reimbursement of our drugs is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

***We are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, which could adversely affect our business, results of operations and financial condition.***

Our operations are subject to anti-corruption laws, including the FCPA, the Bribery Act, and other anti-corruption laws that apply in countries where we do business and may do business in the future. The FCPA, the Bribery Act, and these other laws generally prohibit us, our officers and our employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. We may in the future operate in jurisdictions that pose a high risk of potential FCPA or Bribery Act violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the FCPA, the Bribery Act, or local anti-corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which our international operations might be subject or the manner in which existing laws might be administered or interpreted.

We are also subject to other laws and regulations governing our international operations, including regulations administered by the governments of the United States, United Kingdom, and authorities in the European Union, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, which we collectively refer to as Trade Control Laws.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the FCPA, the Bribery Act, or other legal requirements, including Trade Control Laws. If we are not in compliance with the FCPA, the Bribery Act, and other anti-corruption laws or Trade Control Laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations and liquidity. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions. Likewise, any investigation of any potential violations of the FCPA, the Bribery Act, other anti-corruption laws or Trade Control Laws by U.S., U.K. or other authorities could also have an adverse impact on our reputation, our business, results of operations and financial condition.

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

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

---

## [Table of Contents](#)

We maintain workers' compensation insurance to cover costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, but this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts, which could adversely affect our business, financial condition, results of operations or prospects. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

***Inadequate funding for the FDA, the SEC and other government agencies 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. 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 drugs 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 agencies, such as the FDA and the SEC, have had to furlough critical 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.

### **Risks Related to Ownership of Our Common Stock**

***Our executive officers, directors and principal stockholders maintain the ability to control all matters submitted to stockholders for approval.***

We believe our executive officers, directors and stockholders which own more than 5% of our outstanding common stock, in the aggregate, beneficially own more than a majority of our capital stock. One of our directors is affiliated with a stockholder who beneficially owns more than 5% of our outstanding common stock. If these stockholders were to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and business affairs. For example, these persons, if they act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire or result in management of our company that other stockholders disagree with.

***A substantial number of shares of our common stock may be sold into the market in the near future, including pursuant to our Sales Agreement with Jefferies or our universal shelf registration statement, which could result in dilution to our stockholders and/or cause the market price of our common stock to drop significantly, even if our business is performing well.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time, subject to certain restrictions under U.S. securities laws. A significant number of our total outstanding shares are restricted from resale but may be sold into the market in the near future. Moreover, holders of a substantial number of shares of our common stock have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders.

In May 2019, we completed a private placement of 5,582,940 shares of our common stock to several accredited institutional investors. We have filed a registration statement covering the resale by these investors of the shares of common stock purchased in the private placement, and have agreed to keep the registration statement effective until the date the shares covered by the registration statement have been sold or can be sold without restriction pursuant to Rule 144 of the Securities Act of 1933, as amended. Furthermore, in July 2018, we registered all shares of common stock that we may issue under our equity compensation plans. Registered shares can be freely sold in the public market, subject only to volume limitations applicable to affiliates.

We currently have on file with the SEC a universal shelf registration statement which allows us to offer and sell registered common stock, preferred stock, debt securities, warrants and/or units from time to time pursuant to one or more offerings at prices and terms to be determined at the time of sale. In July 2019, we entered into an Open Market Sale Agreement<sup>SM</sup>, or Sales Agreement, with

---

## [Table of Contents](#)

Jefferies LLC, or Jefferies, pursuant to which, from time to time, we may offer and sell through Jefferies up to \$50.0 million of the common stock registered under the universal shelf registration statement pursuant to one or more “at the market” offerings. Sales of a substantial number of shares of our common stock, or the perception in the market that holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

Sales of substantial amounts of shares of our common stock or other securities by our stockholders, by Jefferies pursuant to the Sales Agreement, under our universal shelf registration statement or otherwise could also dilute our stockholders.

***If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.***

The trading market for our common stock will rely, in part, on the research and reports that industry or financial analysts publish about us or our business. There can be no assurance that existing analysts will continue to cover us or that new analysts will begin to cover us. There is also no assurance that any covering analyst will provide favorable coverage. A lack of research coverage or adverse coverage may negatively impact the market price of our common stock. In addition, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

***The price of our common stock is volatile and may fluctuate substantially, which could result in substantial losses for purchasers of our common stock.***

Our stock price is volatile. The stock market in general, and the market for biopharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- results of preclinical studies and clinical trials of our product candidates or those of our competitors;
- the success of competitive products or technologies;
- commencement or termination of collaborations;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of health care payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- the entry into significant acquisitions, strategic partnerships or divestitures by us or our competitors;
- significant sales of our common stock, including sales by our directors, executive officers or 5% stockholders;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

---

## [Table of Contents](#)

If any of the foregoing matters were to occur, or if our operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation often has been instituted against that company. Such litigation, if instituted against us, could cause us to incur substantial costs to defend such claims and divert management's attention and resources, which could seriously harm our business, financial condition, results of operations and prospects.

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

Our shares of common stock began trading on the Nasdaq Global Select Market on June 28, 2018. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price for our common stock and thereby affect the ability of our stockholders to sell their shares. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

***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," or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We will remain an EGC until the earlier of: (i) the last day of the fiscal year in which we have total annual gross revenue of \$1.07 billion or more; (ii) the last day of the fiscal year following the fifth anniversary of the date of our initial public offering; (iii) the date on which we have issued more than \$1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the SEC, which means the last day of the first fiscal year in which the market value of our common stock that is held by non-affiliates exceeds \$700 million as of June 30. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not EGCs. These exemptions include:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- reduced disclosure obligations regarding executive compensation;
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved; and
- an exemption from compliance with the requirement of the Public Company Accounting Oversight Board regarding the communication of critical audit matters in the auditor's report on the financial statements.

In addition, the JOBS Act provides that an EGC may take advantage of an extended transition period for complying with new or revised accounting standards. This allows an EGC to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not EGCs.

We are also a smaller reporting company, and we will remain a smaller reporting company until the fiscal year following the determination that our voting and non-voting common shares held by non-affiliates is more than \$250 million measured on the last business day of our second fiscal quarter, or our annual revenues are less than \$100 million during the most recently completed fiscal year and our voting and non-voting common shares held by non-affiliates is more than \$700 million measured on the last business day of our second fiscal quarter. Similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations, such as an exemption from providing selected financial data and an ability to provide simplified executive compensation information and only two years of audited financial statements.

We have elected to take advantage of certain of the reduced reporting obligations. Investors may find our common stock less attractive as a result of our reliance 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.

---

## [Table of Contents](#)

***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 requirements.***

As a public company, and particularly after we are no longer an EGC, we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and rules subsequently implemented by the SEC and the Nasdaq Stock Market have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, which could make it more difficult for us to attract and retain qualified members of our board of directors.

***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 control over financial reporting is necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, is 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, as and when required, conducted in connection with Section 404 of the Sarbanes-Oxley Act, or Section 404, or any subsequent testing by our independent registered public accounting firm, as and when required, may reveal deficiencies in our internal control over financial reporting that are deemed to be significant deficiencies or 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 common stock.

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting, including an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. However, while we remain an EGC, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, continue to 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 neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

***We previously identified a material weakness in our internal control over financial reporting, which has been remediated. If we identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect our business and stock price.***

We previously identified a material weakness in our internal control over financial reporting that was unremediated as of December 31, 2017. Although this material weakness was remediated as of December 31, 2018, we cannot assure that we may not identify another material weakness in the future. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis.

In preparation of our financial statements to meet the requirements of our initial public offering, we determined that a material weakness in our internal control over financial reporting existed during fiscal 2016 and remained unremediated as of December 31, 2017. The material weakness we identified is that we did not design and maintain effective controls and procedures over our accounting for and reporting of the income tax impacts of business combinations. This control deficiency could result in a misstatement of our accounts or disclosures that would result in a material misstatement of our annual or interim condensed consolidated financial statements that would not be prevented or detected, and accordingly, we determined that the control deficiency constitutes a material weakness. The material weakness also resulted in revisions to our previously issued 2016 annual consolidated financial statements, which we concluded were not material to those financial statements, and adjustments to our interim condensed consolidated financial statements for the nine months ended September 30, 2017 before their issuance. Specifically, the material weakness resulted in errors in our accounting for and reporting of income taxes and goodwill in the purchase accounting for a business combination and in subsequent reporting periods.

---

## [Table of Contents](#)

We cannot assure you that the measures we have taken to date, and actions we may take in the future, will be sufficient to prevent or avoid potential future material weaknesses. In addition, neither our management nor an independent registered public accounting firm has performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act because no such evaluation has been required. Had we or our independent registered public accounting firm performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act, additional material weaknesses may have been identified. If we are unable to successfully remediate any future material weaknesses in our internal control over financial reporting, or identify any material weaknesses in the future, or otherwise fail to maintain an effective system of internal controls, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements, investors may lose confidence in our financial reporting, and our share price may decline as a result.

***Provisions in our certificate of incorporation and bylaws and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.***

Provisions in our certificate of incorporation and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called “poison pill,” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our certificate of incorporation or bylaws.

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 in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

***Our certificate of incorporation provides that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders’ ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or stockholders.***

Our certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a breach of fiduciary duty owed by our directors, officers, other employees or stockholders to the company or our stockholders, any action asserting a claim against us arising pursuant to the Delaware General Corporation Law or as to which the Delaware General Corporation Law confers jurisdiction on the Court of Chancery of the State of Delaware, or any action asserting a claim arising pursuant to our certificate of incorporation or our bylaws or governed by the internal affairs doctrine. This provision may limit a stockholder’s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, other employees or other stockholders, which may discourage such lawsuits against us and our directors, officers, other employees or other stockholders. Alternatively, if a court were to find this provision in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition.

---

[Table of Contents](#)

*Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.*

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future, and investors seeking cash dividends should not purchase shares of our common stock.

**Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.**

*Sales of Unregistered Securities*

Set forth below is information regarding shares of common stock issued by us during the three months ended June 30, 2019 that were not registered under the Securities Act of 1933, as amended, or the Securities Act. Included is the consideration, if any, we received for such shares and information relating to the section of the Securities Act or rule of the SEC under which exemption from registration was claimed. The below transaction did not involve any underwriters, underwriting discounts or commissions, or any public offering.

On June 21, 2019, we issued 67,406 shares of common stock in connection with a former employee letter agreement. The shares of common stock issued were exempt from registration pursuant to Section 4(a)(2) of the Securities Act because the issuance did not involve a public offering.

*Use of Proceeds from Initial Public Offering of Common Stock*

On July 2, 2018, we closed our IPO of 9,350,000 shares of common stock at a public offering price of \$13.00 per share, and on July 24, 2018, we issued and sold an additional 364,371 shares of common stock at a price of \$13.00 per share pursuant to the exercise of the underwriters' over-allotment option. The aggregate gross proceeds to us from our IPO, inclusive of the over-allotment exercise, were \$126.3 million. The offer and sale of all of the shares in the offering were registered under the Securities Act pursuant to registration statement on Form S-1 (File No. 333-225368), which was declared effective by the SEC on June 27, 2018. Citigroup Global Markets Inc., Leerink Partners LLC and Evercore Group L.L.C. acted as joint book-running managers for the offering and as representatives of the underwriters. The offering commenced on June 27, 2018 and did not terminate until the sale of all of the shares offered.

Aggregate net proceeds from the offering, inclusive of the proceeds from the over-allotment exercise, were \$113.2 million, after deducting underwriting discounts and commissions of \$8.8 million and offering expenses of \$4.3 million payable by us. None of the underwriting discounts and commissions or offering expenses were paid directly or indirectly to any directors or officers of ours or their associates or to persons owning 10% or more of any class of equity securities or to any affiliates of ours.

As of June 30, 2019, we have used approximately \$63.7 million of the net proceeds to fund the development of MRT5005 and MRT5201, to fund the discovery and additional preclinical research and development of additional product candidates and platform enhancement, and for working capital and other general corporate purposes. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed with the SEC on June 29, 2018 pursuant to Rule 424(b) under the Securities Act.

---

## Table of Contents

### Item 6. Exhibits.

<u>Exhibit Number</u>	<u>Description</u>
10.1	<a href="#"><u>Securities Purchase Agreement, dated April 30, 2019, by and among the Registrant and the other parties thereto (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K filed on May 1, 2019).</u></a>
10.2	<a href="#"><u>Registration Rights Agreement, dated April 30, 2019, by and among the Registrant and the other parties thereto (incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K filed on May 1, 2019).</u></a>
31.1*	<a href="#"><u>Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u></a>
31.2*	<a href="#"><u>Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u></a>
32.1**	<a href="#"><u>Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u></a>
32.2**	<a href="#"><u>Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u></a>
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

\* Filed herewith.

\*\* Furnished herewith.







**CERTIFICATION PURSUANT TO  
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with this Quarterly Report on Form 10-Q of Translate Bio, Inc. (the "Company") for the quarter ended June 30, 2019 as filed with the Securities and Exchange Commission on the date hereof (the "Quarterly Report"), the undersigned, Ronald C. Renaud, Jr., President and Chief Executive Officer, 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 his knowledge on the date hereof:

- (1) The Quarterly 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 Quarterly Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: July 31, 2019

By: \_\_\_\_\_  
/s/ Ronald C. Renaud, Jr.  
**Ronald C. Renaud, Jr.**  
**President and Chief Executive Officer**

**CERTIFICATION PURSUANT TO  
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with this Quarterly Report on Form 10-Q of Translate Bio, Inc. (the "Company") for the quarter ended June 30, 2019 as filed with the Securities and Exchange Commission on the date hereof (the "Quarterly Report"), the undersigned, John R. Schroer, Treasurer and Chief Financial Officer, 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 his knowledge on the date hereof:

- (1) The Quarterly 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 Quarterly Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: July 31, 2019

By: \_\_\_\_\_  
/s/ John R. Schroer  
**John R. Schroer**  
**Treasurer and Chief Financial Officer**