
**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 September 30, 2020

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:

| <u>Title of each class</u> | <u>Trading Symbol(s)</u> | <u>Name of each exchange on which registered</u> |
|---------------------------------|--------------------------|--|
| Common Stock, \$0.001 par value | TBIO | The 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 checked="" type="checkbox"/> |
| Non-accelerated filer | <input 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 November 2, 2020, the registrant had 74,321,827 shares of common stock, \$0.001 par value per share, outstanding.

Table of Contents

| | <u>Page</u> | |
|-----------------|---|----|
| PART I. | FINANCIAL INFORMATION | 1 |
| Item 1. | Financial Statements (Unaudited) | 1 |
| | Condensed Consolidated Balance Sheets | 1 |
| | Condensed Consolidated Statements of Operations | 2 |
| | Condensed Consolidated Statements of Comprehensive Income (loss) | 3 |
| | Condensed Consolidated Statements of Stockholders' Equity | 4 |
| | Condensed Consolidated Statements of Cash Flows | 6 |
| | Notes to Condensed Consolidated Financial Statements (Unaudited) | 7 |
| Item 2. | Management's Discussion and Analysis of Financial Condition and Results of Operations | 25 |
| Item 3. | Quantitative and Qualitative Disclosures About Market Risk | 40 |
| Item 4. | Controls and Procedures | 40 |
| PART II. | OTHER INFORMATION | 41 |
| Item 1. | Legal Proceedings | 41 |
| Item 1A. | Risk Factors | 41 |
| Item 5. | Other Information | 89 |
| Item 6. | Exhibits | 90 |
| | Signatures | 91 |

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical fact, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Quarterly Report on Form 10-Q include, among other things, statements about:

- the impacts of the COVID-19 pandemic;
- the initiation, timing, progress and results of our current and future preclinical studies and clinical trials and our research and development programs;
- our estimates regarding expenses, future revenue, capital requirements and need for additional financing;
- our expectations regarding our ability to fund our operating expenses and capital expenditure requirements with our cash and cash equivalents and the period in which we expect that such cash and cash equivalents will enable us to fund such operating expenses and capital expenditure requirements;
- our plans to develop our product candidates;
- the timing of and our ability to submit applications for, obtain and maintain regulatory approvals for our product candidates;
- the potential advantages of our product candidates;
- the rate and degree of market acceptance and clinical utility of our product candidates;
- our estimates regarding the potential market opportunity for our product candidates;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates;
- our ability to identify additional products, product candidates or technologies with significant commercial potential that are consistent with our commercial objectives;
- the impact of government laws and regulations;
- our competitive position;
- developments relating to our competitors and our industry; and
- our ability to establish collaborations or obtain additional funding.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in the “Risk Factors” section, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Quarterly Report on Form 10-Q and the documents that we reference herein and have filed or incorporated by reference hereto completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Quarterly Report on Form 10-Q are made as of the date hereof, and we do not assume any obligation to update any forward-looking statements except as required by applicable law.

[Table of Contents](#)

This Quarterly Report on Form 10-Q includes certain statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties as well as our own estimates of potential market opportunities. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. Our estimates of the potential market opportunities for our product candidates include several key assumptions based on our industry knowledge, industry publications, third-party research and other surveys, which may be based on a small sample size and may fail to accurately reflect market opportunities. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

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

| | September 30, 2020 | December 31, 2019 |
|--|-----------------------|----------------------|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 674,050 | \$ 84,580 |
| Short-term investments | — | 104,098 |
| Collaboration receivables | 25,139 | 4,596 |
| Prepaid expenses and other current assets | 10,822 | 9,391 |
| Restricted cash | 950 | 950 |
| Total current assets | 710,961 | 203,615 |
| Property and equipment, net | 15,044 | 12,539 |
| Right-of-use assets, net | 75,650 | 10,400 |
| Goodwill | 21,359 | 21,359 |
| Intangible assets, net | 81,280 | 85,536 |
| Other assets | 4,334 | 2,752 |
| Total assets | <u>\$ 908,628</u> | <u>\$ 336,201</u> |
| Liabilities and Stockholders' Equity | | |
| Current liabilities: | | |
| Accounts payable | \$ 6,815 | \$ 15,968 |
| Accrued expenses | 11,766 | 7,072 |
| Current portion of deferred revenue | 93,164 | 18,100 |
| Current portion of operating lease liability | 12,279 | 530 |
| Total current liabilities | 124,024 | 41,670 |
| Contingent consideration | 123,740 | 103,655 |
| Deferred revenue, net of current portion | 242,047 | 25,256 |
| Operating lease liability, net of current portion | 53,151 | 12,084 |
| Total liabilities | <u>542,962</u> | <u>182,665</u> |
| Commitments and contingencies (Notes 3 and 12) | | |
| Stockholders' equity: | | |
| Preferred stock, \$0.001 par value; 10,000,000 shares authorized as of September 30, 2020 and December 31, 2019; no shares issued and outstanding as of September 30, 2020 and December 31, 2019 | — | — |
| Common stock, \$0.001 par value; 200,000,000 shares authorized as of September 30, 2020 and December 31, 2019; 74,251,559 shares and 60,022,067 shares issued and outstanding as of September 30, 2020 and December 31, 2019, respectively | 74 | 60 |
| Additional paid-in capital | 758,314 | 512,231 |
| Accumulated deficit | (392,722) | (359,496) |
| Accumulated other comprehensive income | — | 741 |
| Total stockholders' equity | <u>365,666</u> | <u>153,536</u> |
| Total liabilities and stockholders' equity | <u>\$ 908,628</u> | <u>\$ 336,201</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)

| | Three Months Ended | | Nine Months Ended | |
|--|--------------------|-------------|-------------------|-------------|
| | September 30, | | September 30, | |
| | 2020 | 2019 | 2020 | 2019 |
| Collaboration revenue | \$ 66,446 | \$ 1,266 | \$ 87,420 | \$ 3,914 |
| Operating expenses: | | | | |
| Research and development | 26,344 | 17,295 | 76,785 | 51,343 |
| General and administrative | 9,163 | 6,881 | 25,223 | 21,284 |
| Change in fair value of contingent consideration | 14,190 | (19,834) | 20,085 | (3,243) |
| Impairment of intangible asset | — | 18,559 | — | 18,559 |
| Total operating expenses | 49,697 | 22,901 | 122,093 | 87,943 |
| Income (loss) from operations | 16,749 | (21,635) | (34,673) | (84,029) |
| Other income, net | 595 | 408 | 1,447 | 1,286 |
| Income (loss) before benefit from income taxes | 17,344 | (21,227) | (33,226) | (82,743) |
| Benefit from income taxes | — | — | — | 486 |
| Net income (loss) | \$ 17,344 | \$ (21,227) | \$ (33,226) | \$ (82,257) |
| Net income (loss) per share—basic | \$ 0.24 | \$ (0.41) | \$ (0.51) | \$ (1.69) |
| Weighted average common shares outstanding—basic | 73,183,923 | 51,891,157 | 65,187,435 | 48,574,275 |
| Net income (loss) per share—diluted | \$ 0.23 | \$ (0.41) | \$ (0.51) | \$ (1.69) |
| Weighted average common shares outstanding—diluted | 76,440,293 | 51,891,157 | 65,187,435 | 48,574,275 |

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

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

| | <u>Three Months Ended</u> | | <u>Nine Months Ended</u> | |
|---|---------------------------|----------------------|--------------------------|----------------------|
| | <u>September 30,</u> | <u>September 30,</u> | <u>September 30,</u> | <u>September 30,</u> |
| | <u>2020</u> | <u>2019</u> | <u>2020</u> | <u>2019</u> |
| Net income (loss) | \$17,344 | \$(21,227) | \$(33,226) | \$(82,257) |
| Other comprehensive income (loss): | | | | |
| Unrealized gains (losses) on available-for-sale securities, net of tax of \$0 | (540) | 109 | (741) | 483 |
| Comprehensive income (loss) | <u>\$16,804</u> | <u>\$(21,118)</u> | <u>\$(33,967)</u> | <u>\$(81,774)</u> |

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

TRANSLATE BIO, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (UNAUDITED)
(In thousands, except share amounts)

| | Common Stock | | Additional Paid-in Capital | Accumulated Deficit | Accumulated Other Comprehensive Income | Total Stockholders' Equity |
|--|-------------------|--------------|----------------------------------|------------------------|---|----------------------------------|
| | Shares | Amount | | | | |
| Balances at December 31, 2019 | 60,022,067 | \$ 60 | \$512,231 | \$ (359,496) | \$ 741 | \$ 153,536 |
| Exercise of stock options | 15,596 | — | 132 | — | — | 132 |
| Stock-based compensation expense | — | — | 3,172 | — | — | 3,172 |
| Unrealized gains on available-for-sale securities | — | — | — | — | 114 | 114 |
| Net loss | — | — | — | (14,282) | — | (14,282) |
| Balances at March 31, 2020 | 60,037,663 | 60 | 515,535 | (373,778) | 855 | 142,672 |
| Issuance of common stock in connection with public offerings, net of underwriting discounts and commissions and offering costs | 8,544,982 | 9 | 153,602 | — | — | 153,611 |
| Exercise of stock options | 776,864 | — | 5,699 | — | — | 5,699 |
| Stock-based compensation expense | — | — | 6,014 | — | — | 6,014 |
| Unrealized losses on available-for-sale securities | — | — | — | — | (315) | (315) |
| Net loss | — | — | — | (36,288) | — | (36,288) |
| Balances at June 30, 2020 | 69,359,509 | 69 | 680,850 | (410,066) | 540 | 271,393 |
| Issuance of common stock in connection with Securities Purchase Agreement | 4,884,434 | 5 | 73,749 | — | — | 73,754 |
| Exercise of stock options | 7,616 | — | 60 | — | — | 60 |
| Stock-based compensation expense | — | — | 3,655 | — | — | 3,655 |
| Unrealized losses on available-for-sale securities | — | — | — | — | (540) | (540) |
| Net income | — | — | — | 17,344 | — | 17,344 |
| Balances at September 30, 2020 | <u>74,251,559</u> | <u>\$ 74</u> | <u>\$758,314</u> | <u>\$ (392,722)</u> | <u>\$ —</u> | <u>\$ 365,666</u> |

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

TRANSLATE BIO, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (UNAUDITED)
(In thousands, except share amounts)

| | Common Stock | | Additional Paid-in Capital | Accumulated Deficit | Accumulated Other Comprehensive Income | Total Stockholders' Equity |
|---|-------------------|--------------|----------------------------------|------------------------|---|----------------------------------|
| | Shares | Amount | | | | |
| Balances at December 31, 2018 | 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) |
| Balances at March 31, 2019 | 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 | 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) |
| Balances at June 30, 2019 | 51,010,368 | 51 | 422,309 | (307,233) | 570 | 115,697 |
| Issuance of common stock in connection with public offering, net of underwriting discounts and commissions and offering costs | 9,000,000 | 9 | 84,002 | — | — | 84,011 |
| Forfeited restricted common stock | (449) | — | — | — | — | — |
| Exercise of stock options | 10,806 | — | 84 | — | — | 84 |
| Stock-based compensation expense | — | — | 2,899 | — | — | 2,899 |
| Unrealized gains on available-for-sale securities | — | — | — | — | 109 | 109 |
| Net loss | — | — | — | (21,227) | — | (21,227) |
| Balances at September 30, 2019 | <u>60,020,725</u> | <u>\$ 60</u> | <u>\$509,294</u> | <u>\$ (328,460)</u> | <u>\$ 679</u> | <u>\$ 181,573</u> |

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)

| | Nine Months Ended | |
|---|-------------------|------------------|
| | 2020 | 2019 |
| Cash flows from operating activities: | | |
| Net loss | \$ (33,226) | \$ (82,257) |
| Adjustments to reconcile net loss to net cash provided by (used in) operating activities: | | |
| Depreciation and amortization expense | 6,374 | 2,951 |
| Stock-based compensation expense | 12,841 | 8,408 |
| Impairment of intangible asset | — | 18,559 |
| Change in fair value of contingent consideration | 20,085 | (3,243) |
| Deferred income tax benefit | — | (486) |
| Changes in operating assets and liabilities: | | |
| Collaboration receivables | (20,543) | 125 |
| Prepaid expenses and other assets | (5,766) | (2,293) |
| Right-of-use assets | 1,338 | 357 |
| Long-term prepaid rent | (10,057) | (2,492) |
| Accounts payable | (8,560) | (1,740) |
| Accrued expenses | 4,913 | 2,244 |
| Lease liability | (962) | (270) |
| Deferred revenue | 291,855 | (1,763) |
| Net cash provided by (used in) operating activities | <u>258,292</u> | <u>(61,900)</u> |
| Cash flows from investing activities: | | |
| Purchases of investments | (27,409) | (138,156) |
| Sales and maturities of investments | 130,765 | 116,311 |
| Purchases of property and equipment | (5,434) | (2,241) |
| Net cash provided by (used in) investing activities | <u>97,922</u> | <u>(24,086)</u> |
| Cash flows from financing activities: | | |
| Proceeds from public offerings, net of underwriting discounts and commissions | 154,292 | 84,600 |
| Payments of public offering costs | (681) | (589) |
| Proceeds from Securities Purchase Agreement | 73,754 | — |
| Proceeds from private placement, net of placement agent fees | — | 44,608 |
| Payments of private placement offering costs | — | (477) |
| Proceeds from option exercises | 5,891 | 1,500 |
| Net cash provided by financing activities | <u>233,256</u> | <u>129,642</u> |
| Net increase in cash, cash equivalents and restricted cash: | 589,470 | 43,656 |
| Cash, cash equivalents and restricted cash at beginning of period | 85,530 | 56,224 |
| Cash, cash equivalents and restricted cash at end of period | <u>\$ 675,000</u> | <u>\$ 99,880</u> |
| Cash, cash equivalents and restricted cash at end of period: | | |
| Cash and cash equivalents | \$ 674,050 | \$ 98,930 |
| Restricted cash | 950 | 950 |
| Total cash, cash equivalents and restricted cash at end of period | <u>\$ 675,000</u> | <u>\$ 99,880</u> |
| Supplemental disclosure of non-cash investing and financing activities: | | |
| Purchases of property and equipment included in accounts payable and accrued expenses | \$ 377 | \$ 2 |
| Issuance of common stock in connection with a former employee letter agreement | \$ — | \$ 847 |

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, or to prevent infectious diseases by generating protective immunity. Using its proprietary mRNA therapeutic platform (“MRT platform”), the Company creates mRNA that encodes functional proteins. The Company’s mRNA is designed to be 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 primarily focused on applying its MRT platform to treat pulmonary diseases caused by insufficient protein production or where production of proteins can modify disease. The Company is also pursuing the applicability of its MRT platform for the development of mRNA vaccines for infectious diseases under a collaboration with Sanofi Pasteur Inc. (“Sanofi”), the vaccines global business unit of Sanofi S.A.

The outbreak of a novel strain of coronavirus named SARS-CoV-2 (severe acute respiratory syndrome 2), which causes coronavirus disease (“COVID-19”), has presented a substantial public health and economic challenge around the world and is affecting the Company’s employees, patients, communities and business operations, as well as the U.S. economy and financial markets. While the Company has progressed certain of its preclinical programs, specifically in therapeutics for pulmonary diseases and in vaccine development under its collaboration with Sanofi, as further discussed below, in April 2020, the Company announced that enrollment and dosing had been paused in the ongoing Phase 1/2 clinical trial in patients with cystic fibrosis (“CF”) as a consequence of the COVID-19 pandemic. As discussed below in more details, in September 2020, the Company announced that enrollment and dosing resumed. The full extent to which the COVID-19 pandemic will directly or indirectly impact the Company’s business, results of operations and financial condition will depend on future developments that are highly uncertain and cannot be accurately predicted, including new information that may emerge concerning COVID-19, the actions taken in an effort to contain it or to potentially treat or vaccinate against COVID-19 and the economic impact on local, regional, national and international markets. The Company is actively monitoring this situation and the possible effects on its financial condition, liquidity, operations, suppliers, industry and workforce.

The Company is developing MRT5005 for the treatment of 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 in July 2019, the Company reported interim data from the SAD portion of the clinical trial through one-month follow up post dosing. 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 the mid-dose level. Based on the analysis of the interim results, the Company has amended the clinical trial protocol to include one additional SAD dose group and two additional dose groups in the ongoing multiple-ascending dose (“MAD”) portion of this trial. The Company began dosing patients in the MAD portion of the trial in early 2019. In April 2020, the Company announced that enrollment and dosing had been paused in the ongoing Phase 1/2 clinical trial in patients with CF as a consequence of the COVID-19 pandemic. In September 2020, the Company announced that enrollment and dosing resumed. Due to the uncertain environment associated with the COVID-19 pandemic, the Company is unable to predict the rate of enrollment and timing for reporting data.

The Company is leveraging its lung delivery platform and focusing its preclinical research efforts on identifying lead product candidates for a next-generation CF program, as well as beyond CF in additional pulmonary diseases with unmet medical need, including primary ciliary dyskinesia, idiopathic pulmonary fibrosis and pulmonary arterial hypertension.

The Company has a collaboration with Sanofi to develop infectious disease vaccines using the Company’s mRNA technology. Under the collaboration, the Company and Sanofi will jointly conduct research and development activities to advance mRNA vaccines targeting up to seven infectious disease pathogens (see Note 3). Two of the target pathogens under development are SARS-CoV-2 and influenza. The Company and Sanofi have selected MRT5500 as the lead candidate for a vaccine against SARS-CoV-2. A Phase 1/2 clinical trial to evaluate MRT5500 was anticipated to begin in the fourth quarter of 2020, and is now expected to begin in the first quarter of 2021 due to a delay in the manufacturing of clinical trial material. For the influenza vaccine program, lead lipid nanoparticle/mRNA formulation is being evaluated in preclinical studies to support a clinical proof of technology trial anticipated to begin mid-year 2021.

Table of Contents

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 preparation of the accompanying condensed consolidated financial statements requires the Company to make estimates, judgments and assumptions that may affect the reported amounts of assets, liabilities, equity, revenues and expenses and related disclosure of contingent assets and liabilities. On an ongoing basis the Company evaluates its estimates, judgments and methodologies. The Company bases its estimates on historical experience and on various other assumptions that it believes are reasonable, the results of which form the basis for making judgments about the carrying values of assets, liabilities and equity and the amount of revenues and expenses. The full extent to which the COVID-19 pandemic will directly or indirectly impact the Company's business, results of operations and financial condition, including revenue, expenses, reserves and allowances, manufacturing, clinical trials, research and development costs and employee-related amounts, will depend on future developments that are highly uncertain, including as a result of new information that may emerge concerning COVID-19 and the actions taken in an effort to contain it or to potentially treat or vaccinate against COVID-19, as well as the economic impact on local, regional, national and international customers and markets. The Company has made estimates of the impact of COVID-19 within its financial statements and have determined them to be immaterial. There may be changes to those estimates in future periods. Actual results may differ from these estimates.

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. The accompanying unaudited condensed consolidated balance sheet as of September 30, 2020, the unaudited condensed consolidated statements of operations and of comprehensive income (loss) for the three and nine months ended September 30, 2020 and 2019, the unaudited condensed consolidated statements of stockholders' equity for the three and nine months ended September 30, 2020 and 2019 and the unaudited condensed consolidated statements of cash flows for the nine months ended September 30, 2020 and 2019 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, 2019 has been derived from the Company's audited financial statements for the year ended December 31, 2019. 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 condensed 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, 2019 included in the Company's Annual Report on Form 10-K that was filed with the SEC on March 12, 2020.

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, reflects all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's financial position as of September 30, 2020, the results of its operations for the three and nine months ended September 30, 2020 and 2019, and its cash flows for the nine months ended September 30, 2020 and 2019. The financial data and other information disclosed in these notes related to the three and nine months ended September 30, 2020 and 2019 are also unaudited. The results for the three and nine months ended September 30, 2020 are not necessarily indicative of results to be expected for the year ending December 31, 2020, any other interim periods, or any future year or period.

Sales of Common Stock

On March 13, 2020, the Company filed a universal shelf registration statement on Form S-3 with the SEC (the "2020 Shelf") to register for sale from time to time up to \$350.0 million of common stock, preferred stock, debt securities, warrants and/or units in one of more offerings (File No. 333-237159). This registration statement was declared effective on May 4, 2020.

The Company is party to an Open Market Sale AgreementSM (the "Sales Agreement") with Jefferies LLC ("Jefferies") under which the Company may issue and sell shares of its common stock, from time to time, having an aggregate offering price of up to \$100.0 million.

As of September 30, 2020, the Company has issued and sold an aggregate of 2,863,163 shares of its common stock pursuant to the Sales Agreement, resulting in gross proceeds of \$37.9 million, before deducting commissions of \$1.1 million and other offering

[Table of Contents](#)

expenses of \$0.2 million. There were no shares issued or sold pursuant to the Sales Agreement during the three months ended September 30, 2020. In the future, \$62.1 million of shares of common stock remain available to be sold pursuant to the Sales Agreement, which sales, if any, would be made under the 2020 Shelf.

On June 24, 2020, the Company filed a registration statement on Form S-3ASR, which became automatically effective upon filing with the SEC (File No. 333-239405) (the “June 2020 Registration Statement”). The June 2020 Registration Statement registered for sale from time to time common stock, preferred stock, debt securities, warrants and/or units in one or more offerings. On June 30, 2020, the Company issued and sold 5,681,819 shares of common stock and a stockholder of the Company sold 6,824,992 shares of common stock through a public offering pursuant to the June 2020 Registration Statement. The price to the public was \$22.00 per share, resulting in gross proceeds to the Company of \$125.0 million, before deducting underwriting discounts and commissions of \$7.5 million and other offering expenses of \$0.5 million. The Company did not receive any proceeds from the sale of shares of common stock by the stockholder.

Sanofi Pasteur Collaboration and Licensing Agreement

In 2018, the Company entered into a collaboration and license agreement with Sanofi (the “Original Sanofi Agreement”) to develop mRNA vaccines for up to five infectious disease pathogens (the “Licensed Fields”). On March 26, 2020, the Company and Sanofi amended the Original Sanofi Agreement (the “First Sanofi Amendment”) to include vaccines against SARS-CoV-2 as an additional Licensed Field, increasing the number of infectious disease pathogens to up to six. On June 22, 2020, the Company and Sanofi agreed to further amend the Original Sanofi Agreement to expand the scope of the collaboration and licenses granted to Sanofi (the “Second Sanofi Amendment”) (see Note 3). The Original Sanofi Agreement, as amended by the First Sanofi Amendment and the Second Sanofi Amendment, is referred to as the “Amended Sanofi Agreement.”

Pursuant to the Amended Sanofi Agreement, the Company and Sanofi will jointly conduct research and development activities to advance mRNA vaccines targeting up to seven infectious disease pathogens. The term of the research collaboration expires in June 2022 with an option for Sanofi to extend for one additional year. If Sanofi elects to extend the collaboration, the collaboration may be further expanded to jointly conduct research and development activities to advance mRNA vaccines for up to an additional three infectious disease pathogens, bringing the total to up to ten pathogens.

Under the terms of the Amended 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 infectious disease pathogen, with certain specified exceptions.

Pursuant to the Second Sanofi Amendment, Sanofi paid the Company an additional upfront payment of \$300.0 million in August 2020. Additionally, in connection with the execution of the Second Sanofi Amendment, the Company and an affiliate of Sanofi (the “Investor”) entered into a securities purchase agreement (the “Securities Purchase Agreement”) for the sale and issuance of 4,884,434 shares of the Company’s common stock to the Investor at a price of \$25.59 per share representing a 50 percent premium to the 20-day moving average share price prior to signing, for an aggregate purchase price of approximately \$125.0 million. The closing of the transaction contemplated by the Securities Purchase Agreement was consummated on July 20, 2020 (see Note 3).

Liquidity

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 September 30, 2020, the Company has funded its operations primarily through sales of equity securities and research and development collaboration agreements. The Company has incurred recurring losses and cash outflows from operations since its inception, including net losses of \$33.2 million and \$82.3 million for the nine months ended September 30, 2020 and 2019, respectively. In addition, the Company had an accumulated deficit of \$392.7 million as of September 30, 2020. The Company expects to continue to generate operating losses for the foreseeable future.

[Table of Contents](#)

As of November 5, 2020, the date of issuance of these unaudited interim condensed consolidated financial statements, the Company expects that its cash and cash equivalents of \$674.1 million as of September 30, 2020 will be sufficient to fund its operating expenses and capital expenditure requirements through 2023. 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.

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, 2019, and the notes thereto, which are included in the Company's Annual Report on Form 10-K. During the nine months ended September 30, 2020, there were no material changes to the Company's significant accounting policies.

Recently Adopted Accounting Pronouncements

In January 2017, the Financial Accounting Standards Board ("FASB") issued ASU No. 2017-04, *Intangibles—Goodwill and Other: Simplifying the Test for Goodwill Impairment (Topic 350)*, 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. The Company adopted this new standard as of the required effective date of January 1, 2020, and its adoption had no impact on the Company's consolidated financial statements.

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 removes the disclosure requirement for the amount and reasons for transfers between Level 1 and Level 2 fair value measurements as well as the process for Level 3 fair value measurements. In addition, the ASU adds the disclosure requirements for changes in unrealized gains and losses included in other comprehensive income (loss) for recurring Level 3 fair value measurements held at the end of the reporting period as well as the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements. The Company adopted this new standard as of the required effective date of January 1, 2020, and its adoption had no impact on the Company's consolidated financial statements.

In November 2018, the FASB issued ASU No. 2018-18, *Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606*. This update provides clarification on the interaction between Accounting Standards Codification ("ASC") 606, *Revenue from Contracts with Customers* ("ASC 606"), and ASC 808, *Collaborative Arrangements* ("ASC 808"), including the alignment of unit of account guidance between the two topics. The Company adopted this new standard as of the required effective date of January 1, 2020, and its adoption had no impact on the Company's consolidated financial statements.

Recently Issued Accounting Pronouncements

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments—Credit Losses (Topic 326): 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, 2023. The Company is currently evaluating the potential impact that the adoption of this new standard will have on its consolidated financial statements and disclosures.

In December 2019, the FASB issued ASU No. 2019-12, *Income Taxes—Simplifying the Accounting for Income Taxes*. This new standard eliminates certain exceptions related to the approach for intraperiod tax allocation, the methodology for calculating income

taxes in an interim period and the recognition of deferred tax liabilities for outside basis differences. The new guidance also simplifies aspects of the accounting for franchise taxes and enacted changes in tax laws or rates and clarifies the accounting for transactions that result in a step-up in the tax basis of goodwill. The standard is effective for annual periods beginning after December 15, 2020 and interim periods within, with early adoption permitted. Adoption of the standard requires certain changes to be made prospectively, with some changes to be made retrospectively. The Company does not expect the adoption of this new standard to have a material impact on its consolidated financial statements.

3. Collaboration Agreement

Sanofi Collaboration and License Agreement

In 2018, the Company and Sanofi entered into the Original Sanofi Agreement to develop mRNA vaccines and an mRNA vaccine platform for up to five infectious disease pathogens. In March 2020, the Company and Sanofi entered into the First Sanofi Amendment to include vaccines against SARS-CoV-2 as an additional Licensed Field, increasing the number of infectious disease pathogens to up to six. In June 2020, the Company and Sanofi entered into the Second Sanofi Amendment, which became effective in July 2020 following early termination of the waiting period under the Hart-Scott Rodino Act, to expand the scope of the collaboration and licenses granted to Sanofi.

Under the terms of the Amended Sanofi Agreement, the Company has agreed to grant 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 infectious disease pathogen, with certain specified exceptions.

Pursuant to the Amended Sanofi Agreement, the Company and Sanofi will jointly conduct research and development activities to advance mRNA vaccines targeting up to seven infectious disease pathogens. The term of the research collaboration (the "Collaboration Term") expires in June 2022, with an option for Sanofi to extend the Collaboration Term for one additional year, followed by a technology transfer to Sanofi. If Sanofi elects to extend the Collaboration Term, the collaboration may be further expanded to jointly conduct research and development activities to advance mRNA vaccines for up to an additional three infectious disease pathogens, bringing the total to up to ten pathogens. In addition to the research and development and technology transfer, the Company is responsible for manufacturing and supplying non-clinical products, related materials and investigational products as required by the collaboration plan. Pursuant to the Amended Sanofi Agreement, the Company and Sanofi agreed to a governance structure to manage the activities under the collaboration. If the Company and Sanofi do not mutually agree on certain decisions, Sanofi will be able to break a deadlock without the Company's consent under certain conditions. The collaboration includes an estimated budget. Sanofi is responsible for paying reimbursable development costs, including the Company's employee costs, manufacturing costs, and out-of-pocket costs paid to third parties, up to a specified amount for each Licensed Field.

The Company and Sanofi retain the rights to perform their respective obligations and exercise their respective rights under the Amended Sanofi Agreement. Sanofi also granted the Company non-exclusive, sublicensable licenses under patent rights claiming certain improvements that Sanofi may make to the technology the Company had 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. 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.

Pursuant to the Original Sanofi Agreement Sanofi paid the Company an upfront payment of \$45.0 million in 2018. Pursuant to the Second Sanofi Amendment, Sanofi paid the Company an additional upfront payment of \$300.0 million in August 2020. If Sanofi chooses to exercise its option to extend the Collaboration Term for an additional year, Sanofi has agreed to pay the Company an additional payment of \$75.0 million. The Amended Sanofi Agreement provides that the Company is eligible to receive aggregate potential payments of up to \$1.9 billion upon the achievement of additional specified development, regulatory, manufacturing and commercialization milestones, inclusive of the fee to exercise the option to extend the Collaboration Term. In particular, the Company is entitled to receive development, regulatory and sales milestone payments of up to \$148.0 million for each Licensed Field, other than the SARS-CoV-2 Licensed Field, development, regulatory and sales milestone payments of up to \$250.0 million in the SARS-CoV-2 Licensed Field, and one-time manufacturing milestone payments of up to \$200.0 million. In addition, the Company is entitled to receive a \$10.0 million milestone payment from Sanofi following completion of the technology and process transfer.

Under the terms of the Amended Sanofi Agreement, Sanofi has also agreed to pay the Company royalties on net sales of mRNA vaccines in the SARS-CoV-2 Licensed Field in accordance with the terms of and at the same high single digits to low teens percentages set forth in the Original Sanofi Agreement, except where such vaccines are provided as a donation or transferred to a third party without any profit margin, in which case the Company will be paid royalties sufficient to cover its royalty obligations.

[Table of Contents](#)

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

In connection with the execution of the Second Sanofi Amendment, the Company and the Investor entered into the Securities Purchase Agreement for the sale and issuance of 4,884,434 shares of the Company's common stock to the Investor at a price of \$25.59 per share representing a 50 percent premium to the 20-day moving average share price prior to signing, for an aggregate purchase price of approximately \$125.0 million. The closing of the transaction contemplated by the Securities Purchase Agreement was consummated in July 2020, following early termination of the waiting period under the Hart-Scott Rodino Act. Pursuant to the terms of the Securities Purchase Agreement, the Investor agreed not to, without the prior written approval of the Company and subject to specified conditions, directly or indirectly acquire shares of the Company's outstanding common stock, make a tender, exchange, or other offer to acquire shares of the Company's outstanding common stock, solicit proxies or consents with respect to any matter, or undertake other specified actions related to the potential acquisition of additional equity interests in the Company (the "Standstill Restrictions"). Further, the Investor agreed not to, and to cause its affiliates not to, sell or transfer the shares without the prior written approval of the Company subject to specified conditions (the "Lock-Up Restrictions"). The Standstill Restrictions terminate 12 months after the closing date. The Lock-Up Restrictions terminate 18 months from the closing date.

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 commercialization in the Licensed Fields. The Company and Sanofi also entered into a separate supply agreement on June 22, 2020, with an effective date of December 20, 2019, governing the terms of the supply of products by the Company (the "Supply Agreement"). Pursuant to the Supply Agreement, the Company has agreed to use commercially reasonable efforts to manufacture and supply Sanofi with non-clinical and clinical supply of products and other research materials in certain Licensed Fields, as set forth in the Amended Sanofi Agreement. Sanofi will pay the Company for the non-clinical and clinical supply at the Company's cost to manufacture plus a specified markup. The Supply Agreement will remain in effect until terminated in accordance with its terms. However, under the Amended Sanofi Agreement, the Company's obligation to manufacture and supply products is limited to a defined duration based on the Licensed Field of the applicable product. The Supply Agreement may be terminated by the mutual consent of the parties. Sanofi may terminate the Supply Agreement for convenience after a specified notice period, or in the event that the Company does not provide the supply in a timely manner. The Company may terminate the Supply Agreement in the event of a breach by Sanofi of its payment obligations and such breach remains uncured for a specified period. As part of the Second Sanofi Amendment, the Company and Sanofi agreed to negotiate in good faith and enter into a further supply agreement in respect of supply of products in the SARS-CoV-2 Licensed Field for use in Phase 3 clinical trials or commercial supply.

Accounting for the Sanofi Collaboration

For accounting purposes, the Company has combined the Amended Sanofi Agreement, Securities Purchase Agreement, and Supply Agreement because the contracts were negotiated as a package with a single commercial objective, the amount of consideration to be paid in one contract depends on the price or performance of the other contracts, and the goods and services promised in the contracts are a single performance obligation in accordance with ASC 606.

The Company accounts for the Amended Sanofi Agreement under ASC 606. In determining the appropriate amount of revenue to be recognized under 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 Amended 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

[Table of Contents](#)

services represent one performance obligation. Additionally, Sanofi's right to exercise its option to extend the research term and have the Company conduct research and development activities to advance mRNA vaccines for up to an additional three infectious disease pathogens were determined at the time of the modification to not represent material rights, based on the criteria of ASC 606, and therefore do not represent a separate performance obligation.

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 progress of 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. The Company has estimated the completion of manufacturing activities to be in 2024.

The Company has accounted for the Second Sanofi Amendment as a modification to the existing agreement and not as a separate agreement because the additional goods and services are not distinct and therefore form a single performance obligation that is partially satisfied at the date of the contract modification. The effect that the modification has on the transaction price, and on the Company's measure of progress toward complete satisfaction of the performance obligation, was recognized as a cumulative catch-up adjustment of \$30.9 million on the modification date.

During the three and nine months ended September 30, 2020, the Company increased the overall transaction price by \$491.8 million and \$534.7 million, respectively. The transaction price as of September 30, 2020 includes the upfront, non-refundable payments of \$345.0 million for the transfer of the combined license, supply and development obligations under the Original Sanofi Agreement and Second Sanofi Amendment, the premium paid in consideration for common stock of \$51.2 million, which represents the excess of the price paid compared to the fair value of the Company's common stock on the closing date, under the Securities Purchase Agreement, an estimated \$71.8 million in reimbursable employee costs, an estimated \$155.7 million in reimbursable development costs including manufacturing costs and out-of-pocket costs paid to third parties and an estimated \$62.0 million in milestone payments. Under ASC 606, at the end of each reporting period, the Company re-evaluates the variable consideration determined using either the expected value or most likely outcome approach and 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. The estimated collaboration budget is consistently re-evaluated and changes to the budget, if any, require approval by the Joint Steering Committee. If an approved change occurs, the Company will re-evaluate the transaction price which could potentially affect the cumulative amount of revenue recognized. As a result of the Second Sanofi Amendment, the Company revised the budget and collaboration plan.

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|-----------------------|-------------------------------------|----------|------------------------------------|---------|
| | 2020 | 2019 | 2020 | 2019 |
| Collaboration revenue | \$ 66,446 | \$ 1,266 | \$87,420 | \$3,914 |

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

| | September 30, 2020 | December 31, 2019 |
|----------------------|-----------------------|----------------------|
| Contract liabilities | | |
| Deferred revenue | \$ 335,211 | \$ 43,356 |

Deferred revenue is classified as short-term or long-term in the consolidated balance sheets based on the Company's estimate of revenue that will be recognized within the next twelve months which is determined by the cost-to-cost input method which measures the extent of progress towards satisfying the performance obligation. As of September 30, 2020, the aggregate amount of the transaction price allocated to the remaining performance obligation is estimated to be approximately \$589.2 million, which is expected to be recognized as revenue through 2024. Revenue recognized from contract liabilities at the beginning of the period was \$3.5 million and \$1.8 million during the nine months ended September 30, 2020 and 2019, respectively.

4. Intangible Assets and Goodwill

Acquisition of Shire's MRT Program

In December 2016, the Company entered into an asset purchase agreement (as amended in June 2018) 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 program.

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 | September 30, 2020 | | | Net Carrying Amount |
|-------------------------------------|----------------|-----------------------|--------------------------|-------------------|---------------------|
| | | Gross Carrying Amount | Accumulated Amortization | Impairment Charge | |
| (In thousands) | | | | | |
| Definite-lived intangible assets: | | | | | |
| MRT | 6 years | \$ 45,992 | \$ (7,003) | \$ — | \$ 38,989 |
| Indefinite-lived intangible assets: | | | | | |
| IPR&D - CF | Indefinite | 42,291 | — | — | 42,291 |
| Total intangible assets, net | | <u>\$ 88,283</u> | <u>\$ (7,003)</u> | <u>\$ —</u> | <u>\$ 81,280</u> |

| | Estimated Life | December 31, 2019 | | | Net Carrying Amount |
|-------------------------------------|----------------|-----------------------|--------------------------|--------------------|---------------------|
| | | Gross Carrying Amount | Accumulated Amortization | Impairment Charge | |
| (In thousands) | | | | | |
| Definite-lived intangible assets: | | | | | |
| MRT | 8 years | \$ 45,992 | \$ (2,747) | \$ — | \$ 43,245 |
| Indefinite-lived intangible assets: | | | | | |
| IPR&D - CF | Indefinite | 42,291 | — | — | 42,291 |
| IPR&D - OTC | Indefinite | 18,559 | — | (18,559) | — |
| Total intangible assets, net | | <u>\$ 106,842</u> | <u>\$ (2,747)</u> | <u>\$ (18,559)</u> | <u>\$ 85,536</u> |

Table of Contents

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, ornithine transcarbamylase (“OTC”) deficiency and other potential rare diseases. As of the date of acquisition, the IPR&D was determined to be indefinite-lived.

Upon commencement of the Original 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 the intangible asset’s estimated life based on an economic consumption model. The Company recorded amortization expense of \$0 and \$0.4 million during the three months ended September 30, 2020 and 2019, respectively, and \$4.3 million and \$1.2 million during the nine months ended September 30, 2020 and 2019, respectively, related to the definite-lived MRT intangible asset. The estimated aggregate amortization expense for each of the five succeeding fiscal years is \$7.5 million, \$10.6 million, \$11.3 million, \$11.2 million and \$2.7 million for the years ending December 31, 2020, 2021, 2022, 2023 and 2024, 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. The Company determined that the discontinuation of the development of MRT5201 in September 2019 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 determined that there was no residual value to the indefinite-lived IPR&D related to the OTC deficiency program and, as a result, the Company recorded an impairment charge of \$18.6 million during the three months ended September 30, 2019, representing the entire value of the indefinite-lived IPR&D related to the OTC deficiency program. Concurrent with the impairment charge, the Company removed the contingent consideration liability related to this program.

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 nine months ended September 30, 2020. 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. During the nine months ended September 30, 2020 and 2019, the Company did not recognize any impairment charges related to goodwill.

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 September 30, 2020 Using: | | | |
|------------------------------|--|-------------------|-------------------|-------------------|
| | Level 1 | Level 2 | Level 3 | Total |
| Assets: | | | | |
| Money market funds | \$ — | \$ 636,615 | \$ — | \$ 636,615 |
| U.S. government agency bonds | — | — | — | — |
| | <u>\$ —</u> | <u>\$ 636,615</u> | <u>\$ —</u> | <u>\$ 636,615</u> |
| Liabilities: | | | | |
| Contingent consideration | \$ — | \$ — | \$ 123,740 | \$ 123,740 |
| | <u>\$ —</u> | <u>\$ —</u> | <u>\$ 123,740</u> | <u>\$ 123,740</u> |
| | | | | |
| | Fair Value Measurements as of December 31, 2019 Using: | | | |
| | Level 1 | Level 2 | Level 3 | Total |
| Assets: | | | | |
| Money market funds | \$ — | \$ 56,591 | \$ — | \$ 56,591 |
| U.S. government agency bonds | — | 104,098 | — | 104,098 |
| | <u>\$ —</u> | <u>\$ 160,689</u> | <u>\$ —</u> | <u>\$ 160,689</u> |
| Liabilities: | | | | |
| Contingent consideration | \$ — | \$ — | \$ 103,655 | \$ 103,655 |
| | <u>\$ —</u> | <u>\$ —</u> | <u>\$ 103,655</u> | <u>\$ 103,655</u> |

[Table of Contents](#)

During the nine months ended September 30, 2020 and the year ended December 31, 2019, there were no transfers between Level 1, Level 2 and Level 3.

Cash equivalents as of September 30, 2020 and December 31, 2019 consisted of money market funds totaling \$636.6 million and \$56.6 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 December 31, 2019 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.

Valuation of Contingent Consideration

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

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):

| | <u>Unobservable Inputs</u> Projected Year of Payment | <u>Fair Value at</u> | |
|--------------------|--|-------------------------------------|------------------------------------|
| | | <u>September 30,</u> <u>2020</u> | <u>December 31,</u> <u>2019</u> |
| Earnout payments | 2026 - 2039 | 115,079 | \$ 96,097 |
| Milestone payments | 2026 - 2032 | 8,661 | 7,558 |
| | | <u>\$ 123,740</u> | <u>\$ 103,655</u> |

The discount rate used in the third-party valuation was 12.5% and 13.5% as of September 30, 2020 and December 31, 2019, respectively.

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

| | |
|--|---------------------------------------|
| Balance as of December 31, 2019 | <u>Fair Value</u> <u>\$103,655</u> |
| Increase in fair value of contingent consideration | <u>20,085</u> |
| Balance as of September 30, 2020 | <u>\$123,740</u> |

The increase in the fair value of contingent consideration was primarily due to the time value of money due to the passage of time and a decrease in the discount rate.

6. Property and Equipment, Net

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

| | September 30, 2020 | December 31, 2019 |
|---|-----------------------|----------------------|
| Laboratory equipment | \$ 11,763 | \$ 9,044 |
| Computer equipment | 892 | 779 |
| Office equipment | 942 | 883 |
| Leasehold improvements | 5,730 | 5,635 |
| Construction in progress | 5,094 | 3,460 |
| | <u>24,421</u> | <u>19,801</u> |
| Less: Accumulated depreciation and amortization | (9,377) | (7,262) |
| | <u>\$ 15,044</u> | <u>\$ 12,539</u> |

Depreciation and amortization expense related to property and equipment was \$0.8 million and \$0.6 million during the three months ended September 30, 2020 and 2019, respectively, and \$2.1 million and \$1.8 million during the nine months ended September 30, 2020 and 2019, respectively.

7. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

| | September 30, 2020 | December 31, 2019 |
|--|-----------------------|----------------------|
| Accrued employee compensation and benefits | \$ 4,918 | \$ 3,547 |
| Accrued external research and development expenses | 2,058 | 1,763 |
| Accrued consultant and professional fees | 1,358 | 1,390 |
| Other | 3,432 | 372 |
| | <u>\$ 11,766</u> | <u>\$ 7,072</u> |

Included in other accrued expenses as of September 30, 2020 are costs related to entering into the Second Sanofi Agreement.

8. Incentive Stock Options and Restricted Stock

2018 Equity Incentive Plan

On March 7, 2018, the Company's Board of Directors (the "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 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 Board of Directors. As of December 31, 2019, there were 4,829,847 shares of common stock reserved for issuance under the 2018 Plan. On January 1, 2020, the number of shares of common stock that may be issued under the 2018 Plan increased by 2,400,829 shares of common stock. During the nine months ended September 30, 2020, a total of 99,329 shares issued under the 2016 Plan have been cancelled and rolled over to the 2018 Plan, such that there is a total of 7,330,005 shares of common stock reserved for issuance under the 2018 Plan as of September 30, 2020. 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.

[Table of Contents](#)

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 Board of Directors, subject to stockholder approval, adopted, and on June 15, 2018, the Company's 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 Board of Directors. In December 2019, the Board of Directors elected to add no shares of common stock to the 2018 ESPP. As of September 30, 2020, 870,096 shares of common stock were reserved for issuance under this plan.

2016 Stock Incentive Plan

The 2016 Plan provided 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.

Stock Options

The following table summarizes the Company's stock option activity since December 31, 2019 (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, 2019 | 8,646,378 | \$ 8.06 | 8.42 | \$ 3,687 |
| Granted | 2,992,734 | \$ 9.22 | | |
| Exercised | (800,076) | \$ 7.36 | | |
| Forfeited | (529,492) | \$ 8.59 | | |
| Outstanding as of September 30, 2020 | <u>10,309,544</u> | \$ 8.42 | 8.04 | \$55,026 |
| Exercisable as of September 30, 2020 | 4,915,617 | \$ 7.96 | 7.23 | \$27,795 |
| Vested and expected to vest as of September 30, 2020 | 10,309,544 | \$ 8.42 | 8.04 | \$55,026 |

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 nine months ended September 30, 2020 and 2019 was \$10.7 million and \$0.6 million, respectively.

[Table of Contents](#)

The weighted average grant-date fair value per share of stock options granted was \$5.65 and \$5.58 during the nine months ended September 30, 2020 and 2019, respectively.

Stock Option Valuation

The fair value of stock option grants is estimated using the Black-Scholes option-pricing model. The Company completed its initial public offering in July 2018 and therefore lacks company-specific historical and implied volatility information before that date. 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:

| | Nine Months Ended September 30, | |
|--------------------------|------------------------------------|-------|
| | 2020 | 2019 |
| Risk-free interest rate | 0.77% | 2.39% |
| Expected term (in years) | 6.1 | 6.0 |
| Expected volatility | 68.7% | 73.1% |
| Expected dividend yield | 0% | 0% |

Restricted Common Stock

The following table summarizes the Company's restricted stock activity since December 31, 2019:

| | Number of Shares | Weighted Average Grant-Date Fair Value |
|---|---------------------|---|
| Unvested restricted common stock outstanding as of December 31, 2019 | 34,168 | \$ 1.28 |
| Forfeited restricted common stock | — | \$ — |
| Vested restricted common stock | (34,168) | \$ 1.28 |
| Unvested restricted common stock outstanding as of September 30, 2020 | — | \$ — |

Stock-Based Compensation

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|-------------------------------------|-------------------------------------|-----------------|------------------------------------|----------------|
| | 2020 | 2019 | 2020 | 2019 |
| Research and development expenses | \$ 1,578 | \$ 1,301 | \$ 7,123 | \$3,454 |
| General and administrative expenses | 2,077 | 1,598 | 5,718 | 4,954 |
| | <u>\$ 3,655</u> | <u>\$ 2,899</u> | <u>\$12,841</u> | <u>\$8,408</u> |

Included in research and development stock-based compensation expense for the nine months ended September 30, 2020 was \$2.4 million related to the modification of options in connection with the resignation of the Company's former Executive Vice President and Founder ("EVP and Founder"). In connection with this resignation the Company entered into a separation agreement with the EVP and Founder. Under the terms of the separation agreement, vesting of options for the purchase of 176,266 shares of common stock held by the EVP and Founder was accelerated with no change to the exercise price of such options. Stock options for the purchase of 550,278 shares of common stock, representing all of the options held by the EVP and Founder as of the date of his resignation, are exercisable for 18 months following his resignation.

[Table of Contents](#)

As of September 30, 2020, total unrecognized compensation cost related to the unvested stock-based awards was \$27.2 million, which is expected to be recognized over a weighted average period of 2.5 years.

9. Income Taxes

The Company recognized an income tax benefit of \$0 and \$0.5 million during the nine months ended September 30, 2020 and 2019, respectively. There was no income tax benefit recognized during the three months ended September 30, 2020 and 2019. The income tax benefits recognized during the nine months ended September 30, 2019 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.

10. Net Income (Loss) per Share

Basic net loss per share is calculated by dividing net loss attributable to common stockholders by the weighted-average number of common shares outstanding during the period. Diluted net income per share is calculated by dividing the net income attributable to common stockholders by the weighted-average number of common share equivalents outstanding for the period, including any dilutive effect from outstanding stock options.

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|-------------------------------------|-------------|------------------------------------|-------------|
| | 2020 | 2019 | 2020 | 2019 |
| <i>Basic net income (loss) per common share:</i> | | | | |
| Numerator: | | | | |
| Net income (loss) | \$ 17,344 | \$ (21,227) | \$ (33,226) | \$ (82,257) |
| Denominator: | | | | |
| Weighted average common shares outstanding—basic | 73,183,923 | 51,891,157 | 65,187,435 | 48,574,275 |
| Net income (loss) per share—basic | \$ 0.24 | \$ (0.41) | \$ (0.51) | \$ (1.69) |
| <i>Diluted net income (loss) per common share:</i> | | | | |
| Numerator: | | | | |
| Net income (loss) | \$ 17,344 | \$ (21,227) | \$ (33,226) | \$ (82,257) |
| Denominator: | | | | |
| Weighted average common shares outstanding—diluted | 76,440,293 | 51,891,157 | 65,187,435 | 48,574,275 |
| Net income (loss) per share—diluted | \$ 0.23 | \$ (0.41) | \$ (0.51) | \$ (1.69) |

The Company excluded 99,468 and 147,842 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 and nine months ended September 30, 2019, respectively, because those shares had not vested. As of September 30, 2020, there are no unvested shares of restricted common stock.

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:

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|----------------------------------|-------------------------------------|-----------|------------------------------------|-----------|
| | 2020 | 2019 | 2020 | 2019 |
| Options to purchase common stock | — | 8,568,506 | 10,309,544 | 8,568,506 |
| Unvested restricted common stock | — | 72,677 | — | 72,677 |
| | — | 8,641,183 | 10,309,544 | 8,641,183 |

11. Leases

Suite Retention and Development Agreement

In September 2019, the Company entered into a suite retention and development agreement with Albany Molecular Research, Inc. (“AMRI”) under which a series of cleanroom suites were built at AMRI’s manufacturing facility in accordance with the Company’s objectives (“AMRI Agreement”). The AMRI Agreement continues for five years after the build-out is completed, and the Company has the right to extend for an additional three years. The Company has determined this is a lease under ASU No. 2016-02, Leases (Topic 842) (“ASC 842”). Under the AMRI Agreement, the Company will finance \$6.0 million of the costs of the build-out (“Build-Out Costs”). If Build-Out Costs exceed \$6.0 million, the Company and AMRI will share overage costs equally, up to \$11.0 million, and the Company will be responsible for any amounts exceeding \$11.0 million. The Company had paid \$12.8 million towards the Build-Out Costs prior to the lease commencement date which had been recorded within other long-term assets as prepaid rent as these represented payments for lessor owned assets. The Company anticipates making additional payments of \$4.1 million related to the final Build-Out Costs. These costs are included within the right-of-use (“ROU”) assets and lease liabilities recorded at the lease commencement date. Upon the build-out completion date of August 31, 2020 (“AMRI Lease Commencement”), the Company determined that it gained control of the space, in accordance with ASC 842, which resulted in the recording of ROU assets and related lease liabilities of approximately \$66.6 million and \$53.8 million, respectively, with the difference being due to the elimination of previously recorded prepaid rent. As of August 31, 2020, the Company began paying monthly fees of \$1.0 million, which are subject to a 3% increase on January 1 of each calendar year following the first anniversary of the build-out completion. The option to extend the lease for an additional three years was not included in the lease liability as of September 30, 2020 as the Company is not reasonably certain it will exercise this option.

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 September 30, 2020 and December 31, 2019.

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 asset 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 the real estate lease for office and laboratory spaces 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):

| | Nine Months Ended September 30, | |
|--|------------------------------------|-----------------|
| | 2020 | 2019 |
| Lease cost | | |
| Operating lease cost | \$ 3,397 | \$ 2,019 |
| Total lease cost | \$ 3,397 | \$ 2,019 |
| Other information | | |
| Operating cash flows from operating leases | \$ 3,022 | \$ 1,933 |
| Operating lease liabilities arising from obtaining right-of-use assets | \$ 53,778 | — |
| Weighted-average remaining lease term | 5 years | 8 years |
| Weighted-average discount rate | 11.9% | 17.5% |

[Table of Contents](#)

As of September 30, 2020, maturities of operating lease liabilities are as follows (in thousands):

| | <u>September 30, 2020</u> |
|-------------------------------------|---------------------------|
| 2020 | \$ 7,784 |
| 2021 | 14,737 |
| 2022 | 15,178 |
| 2023 | 15,591 |
| 2024 | 16,050 |
| 2025 and thereafter | 19,164 |
| Total future minimum lease payments | 88,504 |
| Less: imputed interest | (23,074) |
| Present value of lease liabilities | <u>\$ 65,430</u> |

As of December 31, 2019, minimum rental commitments under the real estate lease was as follows (in thousands):

| | <u>December 31, 2019</u> |
|-------------------------------------|--------------------------|
| 2020 | \$ 2,659 |
| 2021 | 2,737 |
| 2022 | 2,818 |
| 2023 | 2,860 |
| 2024 | 2,937 |
| 2025 and thereafter | 10,160 |
| Total future minimum lease payments | 24,171 |
| Less: imputed interest | (11,557) |
| Present value of lease liabilities | <u>\$ 12,614</u> |

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 and for all subsequent leases the Company used an appropriate borrowing rate upon commencement date.

12. Commitments and Contingencies

Research, Supply and License Agreements

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 September 30, 2020, the Company's purchase commitments under the agreement totaled \$14.0 million, with \$3.5 million committed as payments each year from 2021 to 2024. Research and development expenses related to this agreement totaled less than \$0.1 million and \$1.8 million during the three months ended September 30, 2020 and 2019, respectively, and \$2.7 million and \$5.4 million during the nine months ended September 30, 2020 and 2019, respectively.

MIT Research Agreement

In September 2019, the Company entered into a research agreement with the Massachusetts Institute of Technology ("MIT") pursuant to which the Company is obligated to reimburse MIT up to \$4.1 million for specified direct and indirect costs to be incurred from January 2020 through December 2022 for specified research activities conducted for the Company (the "2019 MIT Agreement").

[Table of Contents](#)

As of September 30, 2020, the Company paid MIT \$1.4 million towards the total committed amount. Research and development expenses related to this agreement during the three and nine months ended September 30, 2020 were \$0.3 million and \$1.0 million, respectively. There were no amounts payable by the Company under the agreement as of September 30, 2020. The 2019 MIT Agreement expires in December 2022 and may be extended thereafter by mutual agreement of the parties.

MIT Exclusive Patent License Agreement

The Company is a party to at least one 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 certain of the Company’s customized lipid-based nanoparticles used for delivery of coding RNA components in its MRT platform, including products that may be developed under the Company’s collaboration with Sanofi.

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 September 30, 2020 and 2019 and paid \$0.2 million during each of the nine months ended September 30, 2020 and 2019.

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 four years past 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. In 2019, pursuant to such provision, the Company paid \$0.7 million to MIT as MIT’s share of sublicense income with respect to the upfront payment received under the Original Sanofi Agreement. Additionally, the Company will be required to pay MIT a portion of the \$300.0 million upfront payment from Sanofi and a portion of the 50 percent premium payment in consideration for the common stock purchased by the Investor under the Securities Purchase Agreement (see Note 3), as well as future option and milestone payments that the Company may receive pursuant to the Second Sanofi Amendment. The amount payable to MIT is currently being negotiated between the Company and MIT and the final amount payable to MIT is unknown as this time. 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 (see Note 3). The determination of the relative value of such rights is subject to a process described in the Company’s license agreement with MIT.

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.

[Table of Contents](#)

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.

Expenses related to this agreement totaled less than \$0.1 million during each of the three months ended September 30, 2020 and 2019 and \$0.1 million during each of the nine months ended September 30, 2020 and 2019, respectively.

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 September 30, 2020 and December 31, 2019.

Legal Proceedings

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

13. Related Party Transactions

Private Placement

In connection with a private placement of the Company's common stock in May 2019, entities affiliated with Baupost Group, L.L.C. ("Baupost"), 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.

Public Offerings

In connection with a public offering of the Company's common stock in September 2019, Baupost purchased 5,000,000 shares of the Company's common stock at a price per share of \$10.00 for an aggregate purchase price of \$50.0 million.

In connection with a public offering of the Company's common stock in June 2020, Baupost purchased 500,000 shares of the Company's common stock at a price per share of \$22.00 for an aggregate purchase price of \$11.0 million.

14. Subsequent Event

Real Estate Lease

On November 3, 2020, (the "Lease Commencement Date"), the Company entered into a ten-year lease agreement for approximately 138,444 square feet of office and laboratory space located at 200 West Street in Waltham, Massachusetts. The lease agreement includes an extension option of one period of 10 years. The rent commencement date will be the date 12 months after the Lease Commencement Date. Initial base rent shall be \$5.7 million for the first year and approximately \$8.0 million for the second year following the rent commencement date and thereafter shall be subject to a 3% annual increase.

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, 2019, or the 2019 Annual Report, that was filed with the Securities and Exchange Commission, or SEC, on March 12, 2020. This Management's Discussion and Analysis of Financial Condition and Results of Operations contains forward-looking statements that reflect our current plans, estimates, and beliefs. Actual results may differ materially and adversely from those expressed in any forward-looking statements. Among the important factors that could cause actual results to differ materially from those indicated by our forward-looking statements are those discussed under the heading "Risk Factors" in Part II, Item 1A. and elsewhere in this report, and in the 2019 Annual Report.

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, or to prevent infectious diseases by generating protective immunity. Using our proprietary mRNA therapeutic platform, or MRT platform, we create mRNA that encodes functional proteins. Our mRNA is designed to be 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 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. 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, with the potential to transform life-threatening illnesses into manageable chronic conditions. We are primarily focused on applying our MRT platform to treat pulmonary diseases caused by insufficient protein production or where production of proteins can modify disease. We are also pursuing the applicability of our MRT platform for the development of mRNA vaccines for infectious diseases under a collaboration with Sanofi Pasteur Inc., or Sanofi, the vaccines global business unit of Sanofi S.A. We believe our technology is applicable to a broad range of diseases, including diseases that affect the liver.

We are developing MRT5005 for the treatment of cystic fibrosis, or CF. We believe MRT5005 is the first clinical-stage mRNA product candidate designed to deliver mRNA encoding fully functional cystic fibrosis transmembrane conductance regulator, or CFTR, protein to the lung. We have designed MRT5005 to be inhaled via a handheld nebulizer and to be administered in a once-weekly dose. Once the inhaled MRT5005 has entered the epithelial cells lining the patient's lungs, our therapeutic mRNA uses the cells' own machinery for translation and expression of fully functional CFTR protein, thereby restoring this essential ion channel, which we believe will address the pathology of CF directly. Currently approved CFTR modulating therapies are limited to patients with specific genetic mutations; therefore, there remains a significant unmet medical need for patients with CF who have genetic mutations non-amenable to currently approved CFTR modulating therapies. Additionally, patients treated with these current therapies still suffer from a long-term decline in lung function and exacerbations that require hospitalization. MRT5005 is being developed to treat the underlying cause of CF, regardless of the specific genetic mutation, including in patients with limited or no CFTR protein. The U.S. Food and Drug Administration, or FDA, has granted orphan drug designation, fast track designation and rare pediatric disease designation for MRT5005 for the treatment of 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 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, we completed dosing of patients in the single-ascending dose, or SAD, portion of the Phase 1/2 clinical trial and in July 2019, we reported interim data from the SAD portion of the clinical trial through one-month follow up post dosing. 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 the mid-dose level. Based on the analysis of the interim results, we have amended the clinical trial protocol to include one additional SAD dose group and two additional dose groups in the ongoing multiple-ascending dose, or MAD, portion of this trial. We began dosing patients in the MAD portion of this trial in early 2019. In April 2020, we announced that enrollment and dosing had been paused in the ongoing Phase 1/2 clinical trial in patients with CF as a consequence of the coronavirus disease, or COVID-19, pandemic. In September 2020, we announced that enrollment and dosing resumed. Due to the uncertain environment associated with the COVID-19 pandemic, we are unable to predict the rate of enrollment and timing for reporting data.

We are leveraging our lung delivery platform and focusing our preclinical research efforts on identifying lead product candidates for a next-generation CF program, as well as beyond CF in additional pulmonary diseases with unmet medical need. Building upon the MRT5005 program's success to date, we are exploring innovation in the MRT platform including novel lipid nanoparticles, protein engineering approaches and manufacturing process enhancements to identify next-generation CF candidates that can support expansion of our pipeline opportunities. Beyond CF, we have discovery efforts underway to identify lead product candidates in additional pulmonary diseases, including primary ciliary dyskinesia, idiopathic pulmonary fibrosis and pulmonary arterial hypertension.

[Table of Contents](#)

We have also begun to explore ways to apply our mRNA and delivery platform expertise to diseases where the degradation of a protein would lead to therapeutic benefit. We believe that using mRNA to enable the production of a molecule that can help tag a target protein for destruction within the cell may have advantages over other protein degradation approaches, including the ability to reach previously undruggable therapeutic targets and increased target selectivity. We have early discovery efforts ongoing in this area.

Additionally, we intend to leverage the broad applicability of our platform through a collaboration with Sanofi to develop infectious disease vaccines using our mRNA technology. Under the collaboration, we will jointly conduct research and development activities to advance vaccines targeting up to seven infectious disease pathogens. As part of the ongoing vaccine development program, comprehensive *in vivo* studies have been conducted across several infectious disease targets. Multiple development candidates were evaluated against distinct pathogens, all of which were well tolerated across all species tested. Multiple antigens were tested with all demonstrating robust neutralization titers. Two of the target pathogens under development are a novel strain of coronavirus named SARS-CoV-2 (severe acute respiratory syndrome 2), which causes COVID-19, and influenza. After evaluation of multiple COVID-19 vaccine candidates *in vivo* for immunogenicity and neutralizing antibody activity, MRT5500 was selected as the lead candidate for a vaccine against SARS-CoV-2. In October 2020, preclinical data was reported demonstrating that MRT5500 induced potent neutralizing antibodies against SARS-CoV-2 in mice and non-human primates. Two doses of MRT5500 in non-human primates induced neutralizing antibody levels significantly higher than those observed in a panel of samples from COVID-19 patients. It was also demonstrated that MRT5500-immunized mice and non-human primates exhibited a Th1-biased T cell response against SARS-CoV-2. Vaccine-associated enhanced respiratory disease, or VAERD, has generally not been reported to be associated with a Th1-biased T cell response and therefore these data suggest the potential for a reduced risk for VAERD. A Phase 1/2 clinical trial to evaluate MRT5500 was anticipated to begin in the fourth quarter of 2020, and is now expected to begin in the first quarter of 2021 due to a delay in the manufacturing of clinical trial material. For information on risks related to our successful development of a vaccine against COVID-19, please see Part II, Item 1A – “Risk Factors – Risks Related to the COVID-19 Pandemic,” included elsewhere in this Quarterly Report on Form 10-Q. For the influenza vaccine program, lead lipid nanoparticles/mRNA formulation is being evaluated in preclinical studies to support clinical proof of technology trial anticipated to begin mid-year 2021. Preclinical studies are ongoing for targets against additional viral and bacterial pathogens.

The successful development of our product candidates will require, among other things, our mRNA manufacturing capabilities. To date, we have established 100-gram single-batch production with our clinical-stage mRNA therapeutics platform. Build-out of a dedicated manufacturing space through a contract manufacturing partner was completed during the third quarter of 2020 and has the potential to accommodate multiple 250-gram batches per month upon continued investments and third-party supplier arrangements. As it relates to development of a COVID-19 vaccine, depending on the final human COVID-19 vaccine dose and timing of scale-up activities, we estimate that we could have manufacturing capacity to produce 90-360 million doses annually. We plan to further expand our mRNA manufacturing capabilities to increase production capacity, and will need to work with raw material and other third-party suppliers to achieve this goal.

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. Through September 30, 2020, we have funded our operations primarily through sales of equity securities and research and collaboration agreements and we have received proceeds of approximately \$1.1 billion from such transactions.

In July 2019, we entered into an Open Market Sale AgreementSM, or Sales Agreement, with Jefferies LLC, or Jefferies, under which we may issue and sell shares of our common stock, from time to time, having an aggregate offering price of up to \$50.0 million. On March 13, 2020, we amended the Sales Agreement to increase the aggregate dollar amount of shares of common stock that may be sold pursuant to the Sales Agreement from \$50.0 million to \$100.0 million, which became effective when our universal shelf registration statement on Form S-3 (File No. 333-237159), or the 2020 Shelf, was declared effective. As of September 30, 2020, we have issued and sold an aggregate of 2,863,163 shares of our common stock pursuant to the Sales Agreement, resulting in gross proceeds of \$37.9 million, before deducting commissions of \$1.1 million and other offering expenses of \$0.2 million. There were no shares issued or sold pursuant to the Sales Agreement during the three months ended September 30, 2020. In the future, \$62.1 million of shares of common stock remain available to be sold pursuant to the Sales Agreement, which sales, if any, would be made under the 2020 Shelf.

On June 30, 2020, we issued and sold 5,681,819 shares of our common stock through a public offering under a Registration Statement on Form S-ASR, which became automatically effective upon filing on June 24, 2020, at a price per share of \$22.00, resulting in gross proceeds of \$125.0 million, before deducting underwriting discounts and commissions of \$7.5 million and other offering expenses of \$0.5 million.

[Table of Contents](#)

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 \$33.2 million and \$82.3 million for the nine months ended September 30, 2020 and 2019, respectively. As of September 30, 2020, we had an accumulated deficit of \$392.7 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 September 30, 2020, we had cash and cash equivalents of \$674.1 million. We believe that our existing cash and cash equivalents will enable us to fund our operating expenses and capital expenditure requirements through 2023.

Sanofi Pasteur Collaboration and Licensing Agreement

In 2018, we entered into a collaboration and license agreement with Sanofi, or the Original Sanofi Agreement, to develop mRNA vaccines for up to five infectious disease pathogens, or the Licensed Fields. On March 26, 2020, we and Sanofi amended the Original Sanofi Agreement, or the First Sanofi Amendment, to include vaccines against SARS-CoV-2 as an additional Licensed Field, increasing the number of infectious disease pathogens to up to six. On June 22, 2020, we and Sanofi agreed to further amend the Original Sanofi Agreement to expand the scope of the collaboration and licenses granted to Sanofi, or the Second Sanofi Amendment, which closed on July 20, 2020, the effective date. The Original Sanofi Agreement, as amended by the First Sanofi Amendment and the Second Sanofi Amendment, is referred to as the Amended Sanofi Agreement.

Pursuant to the Amended Sanofi Agreement, we and Sanofi will jointly conduct research and development activities to advance mRNA vaccines targeting up to seven infectious disease pathogens. The term of the research collaboration expires in June 2022, with an option for Sanofi to extend for one additional year. If Sanofi elects to extend the collaboration, the collaboration may be further expanded to jointly conduct research and development activities to advance mRNA vaccines for up to an additional three infectious disease pathogens, bringing the total to up to ten pathogens.

Under the terms of the Amended 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 infectious disease pathogen, with certain specified exceptions.

Pursuant to the Second Sanofi Amendment, Sanofi paid us an additional upfront payment of \$300.0 million in August 2020. Additionally, in connection with the execution of the Second Sanofi Amendment, we and an affiliate of Sanofi, or the Investor, entered into a securities purchase agreement, or the Securities Purchase Agreement, for the sale and issuance of 4,884,434 shares of our common stock at a price of \$25.59 per share representing a 50 percent premium to the 20-day moving average share price prior to signing, for an aggregate purchase price of approximately \$125.0 million, which was received in July 2020. The closing of the transaction contemplated by the Securities Purchase Agreement was consummated on July 20, 2020.

Business Impact of the COVID-19 Pandemic

The outbreak of SARS-CoV-2 has presented a substantial public health and economic challenge around the world and is affecting our employees, patients, communities and business operations, as well as the U.S. economy and financial markets. While we have progressed certain of our preclinical programs, specifically in therapeutics for pulmonary diseases and in vaccine development under our collaboration with Sanofi, as further discussed above, in April 2020, we announced that enrollment and dosing had been paused in our Phase 1/2 clinical trial in patients with CF as a consequence of the COVID-19 pandemic. In September 2020, we announced that enrollment and dosing resumed. The full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition will depend on future developments that are highly uncertain and cannot be accurately predicted, including new information that may emerge concerning COVID-19, the actions taken in an effort to contain it or to potentially treat or vaccinate against COVID-19 and the economic impact on local, regional, national and international markets. Management is actively monitoring this situation and the possible effects on our financial condition, liquidity, operations, suppliers, industry and workforce. For additional information on risks posed by the COVID-19 pandemic, please see Part II, Item 1A – “Risk Factors – Risks Related to the COVID-19 Pandemic,” included elsewhere in this Quarterly Report on Form 10-Q.

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

Pursuant to the Amended Sanofi Agreement, we and Sanofi will jointly conduct research and development activities to advance mRNA vaccines targeting up to seven infectious disease pathogens. The term of the research collaboration expires in June 2022, with an option for Sanofi to extend for one additional year. If Sanofi elects to extend the collaboration, the collaboration may be further expanded to jointly conduct research and development activities to advance mRNA vaccines for up to an additional three infectious disease pathogens, bringing the total to up to ten pathogens.

Under the terms of the Amended 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 infectious disease pathogen, with certain specified exceptions.

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. We recognize adjustments in revenue under the cumulative catch-up method. Under this method, the impact of this adjustment on revenue recorded to date is recognized in the period the adjustment is identified.

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;

Table of Contents

- 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 our collaboration with Sanofi;
- costs related to compliance with regulatory requirements; and
- payments made under third-party licensing agreements.

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 September 30, | | Nine Months Ended September 30, | |
|---|-------------------------------------|-----------------|------------------------------------|-----------------|
| | 2020 | 2019 | 2020 | 2019 |
| | (in thousands) | | | |
| Vaccine program | \$ 8,437 | \$ 243 | \$20,412 | \$ 788 |
| MRT5005 program | 2,643 | 5,458 | 12,087 | 17,343 |
| Discovery program | 2,923 | 1,873 | 8,730 | 5,416 |
| MRT5201 program | — | 2,155 | — | 6,241 |
| Oligonucleotide program | — | 107 | — | 202 |
| Unallocated research and development expenses | 12,341 | 7,459 | 35,556 | 21,353 |
| Total research and development expenses | <u>\$26,344</u> | <u>\$17,295</u> | <u>\$76,785</u> | <u>\$51,343</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; expand our manufacturing capabilities; conduct research and development activities to advance mRNA vaccines and develop an mRNA vaccine platform under the Amended Sanofi Agreement; prepare regulatory filings for our product candidates; continue to discover and develop additional product candidates; and potentially advance product candidates from our discovery program into later stages of clinical development. We expect to continue to devote a substantial portion of our resources to our discovery program for the foreseeable future.

[Table of Contents](#)

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, including delays resulting from the COVID-19 pandemic;
- 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 investigational new drug, or 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;
- the success of our collaboration with Sanofi;
- our ability to establish new licensing or collaboration arrangements;
- 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 September 2019, we announced our decision to discontinue the development of MRT5201. 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, such as the recent pause in enrollment and dosing in our ongoing Phase 1/2 clinical trial in patients with CF as a consequence of the COVID-19 pandemic, which has resumed, 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.

[Table of Contents](#)

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, from Shire Human Genetic Therapies, Inc., or Shire, a subsidiary of Takeda Pharmaceutical Company Ltd., we recognized contingent consideration liabilities for future potential milestone and earnout payment obligations. 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.

Impairment of Intangible Assets

In connection with our acquisition of the MRT Program, we recognized indefinite-lived in-process research and development, or IPR&D, which is not subject to amortization, but is tested annually for impairment or more frequently if there are indicators of impairment. Following the impairment test, if the fair value of the indefinite-lived IPR&D is less than its carrying amount, an impairment charge is recognized in the consolidated statements of operations. In September 2019, we announced our decision to discontinue the development of MRT5201 and, as a result, recorded an impairment charge.

Other Income, net

Other income, net primarily consists of income recognized in connection with our investments in money market funds and U.S. government agency bonds.

Benefit from Income Taxes

We recognized an income tax benefit of \$0 and \$0.5 million during the nine months ended September 30, 2020 and 2019, respectively. There was no income tax benefit recognized during the three months ended September 30, 2020 and 2019. The income tax benefits recognized during the nine months ended September 30, 2019 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, 2019, we had U.S. federal net operating loss carryforwards of \$229.3 million, of which \$122.1 million will, if not utilized, begin to expire in 2031. As of December 31, 2019, we had U.S. state net operating loss carryforwards of \$210.6 million, which will, if not utilized, begin to expire in 2031. As of December 31, 2019, we also had U.S. federal and state research and development tax credit carryforwards of \$6.5 million and \$2.7 million, respectively, which will, if not utilized, begin to expire in 2032 and 2028, respectively, and orphan drug tax credit carryforwards of \$13.0 million, which begin to expire in 2037. We also have state investment tax credit carryforwards of \$0.3 million, which will, if not utilized, begin to expire in 2020. As of December 31, 2019, we recorded a full valuation allowance against our deferred tax assets, except for the deferred tax asset associated with our alternative minimum tax credit carryforwards, which will be fully refundable.

Results of Operations

Comparison of the Three Months Ended September 30, 2020 and 2019

The following table summarizes our results of operations for the three months ended September 30, 2020 and 2019:

| | Three Months Ended September 30, | | Change |
|--|-------------------------------------|-------------------|------------------|
| | 2020 | 2019 | |
| | (in thousands) | | |
| Collaboration revenue | \$66,446 | \$ 1,266 | \$ 65,180 |
| Operating expenses: | | | |
| Research and development | 26,344 | 17,295 | 9,049 |
| General and administrative | 9,163 | 6,881 | 2,282 |
| Change in fair value of contingent consideration | 14,190 | (19,834) | 34,024 |
| Impairment of intangible asset | — | 18,559 | (18,559) |
| Total operating expenses | 49,697 | 22,901 | 26,796 |
| Income (loss) from operations | 16,749 | (21,635) | 38,384 |
| Other income, net | 595 | 408 | 187 |
| Income (loss) before benefit from income taxes | 17,344 | (21,227) | 38,571 |
| Benefit from income taxes | — | — | — |
| Net income (loss) | <u>\$17,344</u> | <u>\$(21,227)</u> | <u>\$ 38,571</u> |

Collaboration Revenue

Collaboration revenue was \$66.4 million and \$1.3 million for the three months ended September 30, 2020 and 2019, respectively, which was derived from the Sanofi collaboration. The increase of \$65.2 million was primarily related to increased activities for the vaccine program as well as a \$30.9 million cumulative catch-up of revenue resulting from the Second Sanofi Amendment and the related increase in the transaction price during the three months ended September 30, 2020. See “—Components of Our Results of Operations – Collaboration Revenue” above.

Research and Development Expenses

| | Three Months Ended September 30, | | Change |
|---|-------------------------------------|-----------------|-----------------|
| | 2020 | 2019 | |
| | (in thousands) | | |
| Direct external research and development expenses by program: | | | |
| Vaccine program | \$ 8,437 | \$ 243 | \$ 8,194 |
| MRT5005 program | 2,643 | 5,458 | (2,815) |
| Discovery program | 2,923 | 1,873 | 1,050 |
| MRT5201 program | — | 2,155 | (2,155) |
| Oligonucleotide program | — | 107 | (107) |
| Unallocated research and development expenses: | | | |
| Personnel related (including stock-based compensation) | 6,014 | 4,629 | 1,385 |
| Other | 6,327 | 2,830 | 3,497 |
| Total research and development expenses | <u>\$26,344</u> | <u>\$17,295</u> | <u>\$ 9,049</u> |

Research and development expenses were \$26.3 million for the three months ended September 30, 2020, compared to \$17.3 million for the three months ended September 30, 2019. The increase of \$9.0 million was primarily due to continued development of our vaccine program associated with the Sanofi collaboration and our discovery program as well as an increase in personnel-related costs, partially offset by a decrease in our MRT5005 and MRT5201 programs.

Direct external expenses of our vaccine program increased by \$8.2 million during the three months ended September 30, 2020 compared to the three months ended September 30, 2019 primarily due to increased activities for the vaccine program associated with the Sanofi collaboration.

Direct external expenses of our MRT5005 program decreased by \$2.8 million during the three months ended September 30, 2020 compared to the three months ended September 30, 2019 primarily due to decreased manufacturing and clinical trial costs.

[Table of Contents](#)

Expenses incurred in the three months ended September 30, 2019 related to manufacturing costs in preparation of our Phase 1/2 clinical trial of MRT5005 for the treatment of patients with CF, for which there were no comparable manufacturing expenses in the same period in 2020. The decrease in clinical trial costs in the three months ended September 30, 2020 compared to the same period in 2019 was due to a pause in enrollment and dosing in our ongoing Phase 1/2 clinical trial in patients with CF as a consequence of the COVID-19 pandemic.

Direct external expenses of our discovery program increased by \$1.1 million during the three months ended September 30, 2020 compared to the three months ended September 30, 2019 primarily due to increased costs related to our ongoing exploratory research and discovery efforts to identify next-generation CF candidates and identify lead product candidates in additional pulmonary diseases.

Direct external expenses of our MRT5201 program decreased by \$2.2 million in the three months ended September 30, 2020 compared to the three months ended September 30, 2019 due to the decision in 2019 to discontinue development of this program.

Unallocated research and development expenses increased by \$4.9 million during the three months ended September 30, 2020 compared to the three months ended September 30, 2019. The increase of \$1.4 million in personnel-related costs was primarily related to an increase in headcount in the three months ended September 30, 2020 compared to the same period in 2019. The increase of \$3.5 million in other unallocated research and development expenses was due to an increase of \$2.6 million primarily due to costs related to entering into the Second Sanofi Agreement and an increase of \$1.4 million in occupancy costs, partially offset by a decrease of \$0.4 million in professional fees.

General and Administrative Expenses

General and administrative expenses were \$9.2 million for the three months ended September 30, 2020, compared to \$6.9 million for the three months ended September 30, 2019. The increase of \$2.3 million was primarily due to an increase of \$1.2 million in costs related to entering into the Second Sanofi Amendment and an increase of \$0.8 million in personnel-related costs primarily due to an increase in stock-based compensation expense.

Change in Fair Value of Contingent Consideration

During the three months ended September 30, 2020 and 2019, we recognized operating expenses of \$14.2 million and operating income of \$19.8 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. The expense recognized during the three months ended September 30, 2020 was attributed primarily to an increase in the fair value of the contingent consideration liability for future earnout payments that could become due. This increase was primarily due to the time value of money due to the passage of time and a decrease in the discount rate.

Impairment of Intangible Asset

In September 2019, we announced our decision to discontinue the development of MRT5201. We determined this was an indicator of impairment and, as a result, retested the indefinite-lived IPR&D related to the ornithine transcarbamylase, or OTC, deficiency program for impairment. We determined that there was no residual value to the indefinite-lived IPR&D related to the OTC deficiency program and, as a result, in the three months ended September 30, 2019, we recognized an impairment charge of \$18.6 million representing the entire value of the indefinite-lived IPR&D related to the OTC deficiency program.

Comparison of the Nine Months Ended September 30, 2020 and 2019

The following table summarizes our results of operations for the nine months ended September 30, 2020 and 2019:

| | Nine Months Ended September 30, | | Change |
|--|------------------------------------|--------------------|------------------|
| | 2020 | 2019 | |
| | (in thousands) | | |
| Collaboration revenue | \$ 87,420 | \$ 3,914 | \$ 83,506 |
| Operating expenses: | | | |
| Research and development | 76,785 | 51,343 | 25,442 |
| General and administrative | 25,223 | 21,284 | 3,939 |
| Change in fair value of contingent consideration | 20,085 | (3,243) | 23,328 |
| Impairment of intangible asset | — | 18,559 | (18,559) |
| Total operating expenses | <u>122,093</u> | <u>87,943</u> | <u>34,150</u> |
| Income (loss) from operations | (34,673) | (84,029) | 49,356 |
| Other income, net | 1,447 | 1,286 | 161 |
| Income (loss) before benefit from income taxes | (33,226) | (82,743) | 49,517 |
| Benefit from income taxes | — | 486 | (486) |
| Net loss | <u>\$ (33,226)</u> | <u>\$ (82,257)</u> | <u>\$ 49,031</u> |

Collaboration Revenue

Collaboration revenue was \$87.4 million and \$3.9 million for the nine months ended September 30, 2020 and 2019, respectively, which was derived from the Sanofi collaboration. The increase of \$83.5 million was primarily due to increased activities for the vaccine program during the nine months ended September 30, 2020 as well as a \$30.9 million cumulative catch-up of revenue resulting from the Second Sanofi Amendment and the related increase in the transaction price during the nine months ended September 30, 2020. See “—Components of Our Results of Operations – Collaboration Revenue” above.

Research and Development Expenses

| | Nine Months Ended September 30, | | Change |
|---|------------------------------------|-----------------|-----------------|
| | 2020 | 2019 | |
| | (in thousands) | | |
| Direct external research and development expenses by program: | | | |
| Vaccine program | \$20,412 | \$ 788 | \$19,624 |
| MRT5005 program | 12,087 | 17,343 | (5,256) |
| Discovery program | 8,730 | 5,416 | 3,314 |
| MRT5201 program | — | 6,241 | (6,241) |
| Oligonucleotide program | — | 202 | (202) |
| Unallocated research and development expenses: | | | |
| Personnel related (including stock-based compensation) | 20,792 | 13,615 | 7,177 |
| Other | <u>14,764</u> | <u>7,738</u> | <u>7,026</u> |
| Total research and development expenses | <u>\$76,785</u> | <u>\$51,343</u> | <u>\$25,442</u> |

Research and development expenses were \$76.8 million for the nine months ended September 30, 2020, compared to \$51.3 million for the nine months ended September 30, 2019. The increase of \$25.4 million was primarily due to continued development of our vaccine program associated with the Sanofi collaboration and our discovery program as well as an increase in personnel-related costs, partially offset by a decrease in our MRT5201 and MRT5005 programs.

Direct external expenses of our vaccine program increased by \$19.6 million during the nine months ended September 30, 2020 compared to the nine months ended September 30, 2019 primarily due to increased activities for the vaccine program associated with the Sanofi collaboration.

Direct external expenses of our MRT5005 program decreased by \$5.3 million during the nine months ended September 30, 2020 compared to the nine months ended September 30, 2019 primarily due to decreased manufacturing and clinical trial costs. Expenses incurred in the nine months ended September 30, 2019 related to manufacturing costs in preparation of our Phase 1/2 clinical trial of MRT5005 for the treatment of patients with CF, for which there were no comparable manufacturing expenses in the same period in 2020. The decrease in clinical trial costs during the nine months ended September 30, 2020 compared to the same period in 2019 is due to a pause in enrollment and dosing in our ongoing Phase 1/2 clinical trial in patients with CF as a consequence of the COVID-19 pandemic.

[Table of Contents](#)

Direct external expenses of our discovery program increased by \$3.3 million during the nine months ended September 30, 2020 compared to the nine months ended September 30, 2019 primarily due to increased costs related to our ongoing exploratory research and discovery efforts to identify next-generation CF candidates and identify lead product candidates in additional pulmonary diseases.

Direct external expenses of our MRT5201 program decreased by \$6.2 million in the nine months ended September 30, 2020 compared to the nine months ended September 30, 2019 due to the decision in 2019 to discontinue development of this program.

Unallocated research and development expenses increased by \$14.2 million during the nine months ended September 30, 2020 compared to the nine months ended September 30, 2019. The increase of \$7.2 million in personnel-related costs was primarily related to an increase in stock-based compensation due to a stock option modification in the nine months ended September 30, 2020 as well as an increase in headcount in the nine months ended September 30, 2020 compared to the same period in 2019. The increase of \$7.0 million in other unallocated research and development expenses was due to an increase of \$3.4 million in amortization expense related to the definite-lived MRT intangible asset, an increase of \$2.6 million primarily related to costs associated with entering into the Second Sanofi Agreement and an increase of \$1.5 million in occupancy costs, partially offset by a decrease of \$0.7 million in professional fees.

General and Administrative Expenses

General and administrative expenses were \$25.2 million for the nine months ended September 30, 2020, compared to \$21.3 million for the nine months ended September 30, 2019. The increase of \$3.9 million was due to an increase of \$2.0 million in costs related to entering into the Second Sanofi Amendment and an increase of \$1.4 million in personnel-related costs primarily due to an increase in stock-based compensation expense.

Change in Fair Value of Contingent Consideration

During the nine months ended September 30, 2020 and 2019, we recognized operating expenses of \$20.1 million and operating income of \$3.2 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. The expense recognized during the nine months ended September 30, 2020 was attributed primarily to an increase in the fair value of the contingent consideration liability for future earnout payments that could become due. This increase was primarily due to the time value of money due to the passage of time and a decrease in the discount rate.

Impairment of Intangible Asset

In September 2019, we announced our decision to discontinue the development of MRT5201. We determined this was an indicator of impairment and, as a result, retested the indefinite-lived IPR&D related to the OTC deficiency program for impairment. We determined that there was no residual value to the indefinite-lived IPR&D related to the OTC deficiency program and, as a result, in the nine months ended September 30, 2019, we recognized an impairment charge of \$18.6 million representing the entire value of the indefinite-lived IPR&D related to the OTC deficiency program.

Benefit from Income Taxes

During the nine months ended September 30, 2020 and 2019, we recognized an income tax benefit of \$0 and \$0.5 million, respectively. The income tax benefit recognized during the nine months ended September 30, 2019 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 through September 30, 2020, we have not generated any revenue from product sales 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 condensed consolidated financial statements in Part I, Item 1 of this Quarterly Report on Form 10-Q for a further discussion of our liquidity.

[Table of Contents](#)

Through September 30, 2020, we have funded our operations primarily through sales of equity securities and research and collaboration agreements and we have received proceeds of approximately \$1.1 billion from such transactions.

On March 13, 2020, we filed a universal shelf registration statement on Form S-3 with the SEC, or the 2020 Shelf, to register for sale from time to time up to \$350.0 million of our common stock, preferred stock, debt securities, warrants and/or units in one of more offerings (File No. 333-237159). This registration statement was declared effective on May 4, 2020.

We are party to an Open Market Sale AgreementSM, or Sales Agreement, with Jefferies, under which we may issue and sell shares of our common stock, from time to time, having an aggregate offering price of up to \$100.0 million.

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 shares of our common stock based upon our instructions. We are not obligated to make any sales of our common stock under the Sales Agreement. As of September 30, 2020, we have issued and sold an aggregate of 2,863,163 shares of our common stock, resulting in gross proceeds of \$37.9 million, before deducting commissions of \$1.1 million and other offering expenses of \$0.2 million. In the future, \$62.1 million of shares of common stock remain available to be sold pursuant to the Sales Agreement, which sales, if any, would be made under the 2020 Shelf.

On June 24, 2020, we filed a registration statement on Form S-3ASR, which became automatically effective upon filing with the SEC (File No. 333-239405), referred to as the June 2020 Registration Statement. The June 2020 Registration Statement registered for sale from time to time common stock, preferred stock, debt securities, warrants and/or units in one or more offerings. On June 30, 2020, we issued and sold 5,681,819 shares of common stock and a stockholder sold 6,824,992 shares of common stock through a public offering pursuant to the June 2020 Registration Statement. The price to the public was \$22.00 per share, resulting in gross proceeds to us of \$125.0 million, before deducting underwriting discounts and commissions of \$7.5 million and other offering expenses of \$0.5 million. We did not receive any proceeds from the sales of shares of common stock by the stockholder.

Cash Flows

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

| | <u>Nine Months Ended September 30,</u> | |
|--|--|------------------|
| | <u>2020</u> | <u>2019</u> |
| | <u>(in thousands)</u> | |
| Net cash provided by (used in) operating activities | \$ 258,292 | \$ (61,900) |
| Net cash provided by (used in) investing activities | 97,922 | (24,086) |
| Net cash provided by financing activities | 233,256 | 129,642 |
| Net increase in cash, cash equivalents and restricted cash | <u>\$ 589,470</u> | <u>\$ 43,656</u> |

Operating Activities

During the nine months ended September 30, 2020, operating activities provided \$258.3 million of cash, resulting from net cash provided by changes in our operating assets and liabilities of \$252.2 million and net non-cash charges of \$39.3 million, partially offset by our net loss of \$33.2 million. Net cash provided by changes in our operating assets and liabilities consisted of a \$291.9 million increase in deferred revenue as a result of the Second Sanofi Amendment and a \$4.9 million increase in accrued expenses, partially offset by a \$20.5 million increase in collaboration receivables, a \$10.1 million increase in long-term prepaid rent, an \$8.6 million decrease in accounts payable and a \$5.8 million increase in prepaid expenses and other assets. Net non-cash charges for the nine months ended September 30, 2020 primarily consisted of a \$20.1 million increase in the change in the fair value of contingent consideration which was primarily due to the time value of money due to the passage of time and a decrease in the discount rate, a \$12.8 million charge to stock-based compensation expense and a \$6.4 million charge for depreciation and amortization expense.

During the nine months ended September 30, 2019, operating activities used \$61.9 million of cash, resulting from our net loss of \$82.3 million and net cash used in changes in our operating assets and liabilities of \$5.8 million, partially offset by net non-cash charges of \$26.2 million. Net cash used in changes in our operating assets and liabilities consisted of a \$2.5 million increase in long-term prepaid rent, a \$2.3 million increase in prepaid expenses and other assets and a \$1.7 million decrease in accounts payable. Net non-cash charges for the nine months ended September 30, 2019 primarily consisted of an impairment charge of \$18.6 million representing the entire value of the indefinite-live IPR&D related to the OTC deficiency program resulting from our decision to discontinue the development of MRT5201 and an \$8.4 million increase in stock-based compensation expense. These non-cash expenses were partially offset by a net \$3.2 million decrease in the

[Table of Contents](#)

change in the fair value of contingent consideration which was primarily due to the decision to discontinue the development of MRT5201, which resulted in the removal of the \$23.2 million in contingent consideration liability related to this program, partially offset by an increase in the fair value of contingent consideration due to the continued progress of MRT5005, the time value of money due to the passage of time and a decrease in the discount rate.

Investing Activities

During the nine months ended September 30, 2020, net cash provided by investing activities was \$97.9 million, consisting of \$130.8 million of sales and maturities of short-term investments, partially offset by \$27.4 million of purchases of short-term investments and \$5.4 million of purchases of property and equipment.

During the nine months ended September 30, 2019, net cash used in investing activities was \$24.1 million, consisting of \$138.2 million of purchases of short-term investments and \$2.2 million of purchases of property and equipment, partially offset by \$116.3 million of sales and maturities of short-term investments.

Financing Activities

During the nine months ended September 30, 2020, net cash provided by financing activities was \$233.3 million, consisting of net cash proceeds of \$153.6 million from public offerings of our common stock, \$73.8 million in proceeds, excluding the premium paid in consideration for common stock of \$51.2 million, from the Securities Purchase Agreement entered into with the Investor and \$5.9 million in proceeds from option exercises.

During the nine months ended September 30, 2019, net cash provided by financing activities was \$129.6 million, consisting of net cash proceeds of \$44.1 million from a private placement of our common stock, net cash proceeds of \$84.0 million from a public offering of our common stock and \$1.5 million in 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:

- continue the clinical development of MRT5005;
- continue the development of mRNA vaccine candidates against infectious diseases, including MRT5500, the lead vaccine candidate against SARS-CoV-2;
- 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 manufacturing, operational, financial and management systems;
- 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

[Table of Contents](#)

- incur additional legal, accounting and other expenses in operating as a public company.

We believe that our existing cash and cash equivalents of \$674.1 million as of September 30, 2020 will enable us to fund our operating expenses and capital expenditure requirements through 2023. 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.

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 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 impacts of the COVID-19 pandemic and our response to it;
- 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;
- 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; and
- the timing, receipt and amount of sales of, or milestone payments related to or royalties on, our current or future product candidates, if any.

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.

[Table of Contents](#)

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.

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 grant licenses on terms that may not be favorable to us.

Contractual Obligations and Commitments

During the nine months ended September 30, 2020, there were no material changes to our contractual obligations and commitments as of December 31, 2019 described under Management's Discussion and Analysis of Financial Condition and Results of Operations in our 2019 Annual Report with the exception of the commitments as described below.

On March 26, 2020, we and Sanofi entered into the First Sanofi Amendment to include vaccines against SARS-CoV-2 as an additional Licensed Field, increasing the number of infectious disease pathogens to up to six. Pursuant to the First Sanofi Amendment, we and Sanofi agreed that no upfront fee is payable by Sanofi to us with respect to the addition of SARS-CoV-2 as a Licensed Field. We and Sanofi also agreed that certain provisions of the Original Sanofi Agreement, including provisions related to milestone payments, royalties and royalty reductions, shall not apply to vaccine products for the prevention, treatment or cure of SARS-CoV-2 that are purchased by a governmental authority while SARS-CoV-2 is a declared pandemic. We and Sanofi agreed to negotiate in good faith the royalty terms applicable to such products, which terms shall reflect the economic conditions applicable to commercializing such products and shall not exceed the royalty terms for the existing Licensed Fields. On June 22, 2020, we and Sanofi entered into the Second Sanofi Amendment, which further amends the Original Sanofi Agreement to expand the scope of the collaboration and licenses granted to Sanofi, which became effective on July 20, 2020.

In connection with the execution of the Second Sanofi Amendment, we and Sanofi also entered into the Supply Agreement with an effective date of December 20, 2019, governing the terms of the supply of products by us to Sanofi. Pursuant to the Supply Agreement, we have agreed to use commercially reasonable efforts to manufacture and supply Sanofi with non-clinical and clinical supply of products and other research materials in certain Licensed Fields, as set forth in the Second Sanofi Amendment.

Under our license agreement with the Massachusetts Institute of Technology, or MIT, we are 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 our first commercial sale of each licensed product, and to pay royalties of a low single-digit percentage to MIT based on our, and any of our affiliates' and sublicensees', net sales of licensed products. As a result of the Amended Sanofi Agreement, we will be required to pay MIT a portion of the \$300.0 million upfront payment and a portion of the 50 percent premium payment in consideration for the common stock purchased under the Securities Purchase Agreement as well as future option and milestone payments that we may receive pursuant to the Second Sanofi Amendment. The amount payable to MIT is currently being negotiated between us and MIT and the final amount payable to MIT is unknown as this time. The amounts that we may owe to MIT will depend upon the relative value of the patents we licensed from MIT and sublicensed to Sanofi as compared to the other rights that we licensed to Sanofi. The determination of the relative value of such rights is subject to a process described in our license agreement with MIT.

Critical Accounting Policies and Significant Judgments and Estimates

Our condensed consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our condensed consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses, and the disclosure of contingent assets and liabilities in our finance statements. 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 our 2019 Annual Report.

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.

On June 30, 2020, the market value of our stock held by non-affiliates was greater than \$700 million. As a result, we will cease being an emerging growth company and a smaller reporting company effective December 31, 2020 and will no longer be able to take advantage of the various reporting and other exemptions available to emerging growth companies as of such date. As of the date we file our first Quarterly Report on Form 10-Q following December 31, 2020, we will no longer be able to take advantage of the various reporting and other exemptions to smaller reporting 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 September 30, 2020. 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 September 30, 2020.

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 September 30, 2020 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 the COVID-19 Pandemic

The COVID-19 pandemic has adversely disrupted, and is expected to continue to adversely disrupt, our operations, including our ability to complete our ongoing clinical trials, and may have other adverse effects on our business and operations. In addition, this pandemic has caused substantial disruption in the financial markets and may adversely impact economies worldwide, both of which could result in adverse effects on our business, operations and ability to raise capital.

In December 2019, SARS-CoV-2, a novel strain of a virus that causes COVID-19, surfaced in Wuhan, China and has since spread world-wide. The World Health Organization has declared the outbreak of COVID-19 a global pandemic. The COVID-19 pandemic is evolving, and to date has led to the implementation of various responses, including government-imposed quarantines, travel restrictions and other public health safety measures. The pandemic and government measures taken in response have also had a significant impact, both direct and indirect, on business and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production have been suspended; and demand for certain goods and services, such as medical services and supplies, has spiked, while demand for other goods and services, such as travel, has fallen. The extent to which the ongoing COVID-19 pandemic impacts our operations or those of the third parties on which we rely will depend on many factors, which are highly uncertain and cannot be predicted with confidence, including the duration of the pandemic, additional or modified government actions, new information that may emerge concerning the severity and impact of COVID-19, and the actions to contain COVID-19 or address its impact in the short and long term.

In April 2020, we announced that enrollment and dosing were paused in our ongoing Phase 1/2 clinical trial of MRT5005 in patients with CF as a consequence of the COVID-19 pandemic. In September 2020, we announced that enrollment and dosing resumed. Due to the uncertain environment associated with the COVID-19 pandemic, we are unable to predict the rate of enrollment and timing for reporting data. Further, in response to the COVID-19 pandemic and in accordance with direction from state and local governmental authorities, we have restricted access to our facility to those individuals who perform critical research, translational medicine and laboratory support activities that must be completed on site, limited the number of such people that can be present at our facility at any one time and required that our non-laboratory personnel work remotely. In the event that governmental authorities were to keep these restrictions in place for an extended period or impose further restrictions, our employees conducting research and development activities may not be able to access our laboratory space, and our core research activities may be significantly limited or curtailed, possibly for an extended period of time. The COVID-19 pandemic may further delay or otherwise adversely affect our clinical development activities as a result of many factors, including:

- diversion of healthcare resources away from the conduct of our clinical trials in order to focus on pandemic concerns, including the availability of necessary materials, the attention of physicians serving as our clinical trial investigators, access to hospitals serving as our clinical trial sites, and availability of hospital staff supporting the conduct of our clinical trials;
- potential interruptions in global shipping affecting the transport of clinical trial materials, such as investigational drug product, patient samples, and other supplies used in our clinical trials;
- the impact of further limitations on travel that could interrupt key clinical trial activities, such as clinical trial site initiations and monitoring activities, travel by our employees, contractors or patients to clinical trial sites, or the ability of employees at any of our contract manufacturers or contract research organizations to report to work, any of which could delay or adversely impact the conduct or progress of our clinical trials for MRT5005 and other research and manufacturing activities, and limit the amount of clinical data we will be able to report;

Table of Contents

- any future interruption of, or delays in receiving, supplies of clinical trial material from our contract manufacturing organizations due to staffing shortages, production slowdowns or stoppages, or disruptions in delivery systems;
- availability of future capacity at our contract manufacturers to produce sufficient drug substance and drug product to meet forecasted clinical trial demand if any of these manufacturers elect or are required to divert attention or resources to the manufacture of other pharmaceutical products;
- delays in ongoing laboratory experiments and operations if we are required to further reduce the number of employees in our laboratories, or if the contract research organizations, or CROs, we have retained to supplement our internal research efforts are unable to perform as anticipated, whether due to capacity constraints, staffing shortages, or otherwise; and
- business disruptions caused by potential workplace closures and an increased reliance on employees working from home, challenges in recruiting employees required to execute on our research and development plans, cybersecurity and data accessibility issues, and communication or transit disruptions, any of which could adversely impact our business operations and delay necessary interactions among our employees and between our company and the third parties upon which we rely.

Any negative impact that the COVID-19 pandemic has on recruiting or retaining patients in our clinical trials for MRT5005, the ability of our suppliers to provide materials for our product candidates, or the regulatory review process could cause additional delays with respect to product development activities, which could materially and adversely affect our ability to obtain regulatory approval for and to commercialize our product candidates, increase our operating expenses, affect our ability to raise additional capital, and have a material adverse effect on our financial results.

The response to the COVID-19 pandemic may redirect resources with respect to regulatory and intellectual property matters in a way that would adversely impact our ability to progress regulatory approvals and protect our intellectual property. In addition, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions.

The COVID-19 pandemic continues to rapidly evolve and its ultimate scope, duration and effects are unknown. The extent of the impact of the disruptions to our business, preclinical studies and clinical trials as a result of the COVID-19 pandemic will depend on future developments, which are highly uncertain and cannot be predicted with confidence, such as the ultimate geographic spread of the disease, the duration of the outbreak, travel restrictions and actions to contain the outbreak or treat its impact, such as social distancing and quarantines or lock-downs in the United States and other countries, business closures or business disruptions and the effectiveness of actions taken in the United States and other countries to contain and treat the disease.

The pandemic has already caused significant disruptions in the financial markets, and may continue to cause such disruptions, which could adversely impact any future plans to raise capital and may also impact the volatility of our stock price and trading in our stock. Moreover, it is possible the pandemic will significantly impact economies worldwide, which could result in adverse effects on our business and operations. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business and it has the potential to adversely affect our business, financial condition, results of operations, and prospects.

We and Sanofi may not be successful in our joint efforts to successfully develop in an expedited timeframe an mRNA vaccine against SARS-CoV-2, the virus responsible for COVID-19.

In 2018, we entered into a collaboration with Sanofi Pasteur Inc., or Sanofi, the vaccines global business unit of Sanofi S.A., to develop infectious disease vaccines using our messenger RNA, or mRNA, technology. In March 2020, we and Sanofi amended this collaboration to include mRNA vaccines against SARS-CoV-2, the virus responsible for COVID-19, as an additional infectious disease pathogen and in July 2020, we and Sanofi further expanded our collaboration to develop mRNA vaccines for additional infectious diseases. Pursuant to the amended collaboration, we are leveraging our mRNA platform and Sanofi's vaccine expertise to develop mRNA vaccines for infectious diseases, which includes a goal of discovering, developing and advancing rapidly into the clinic a SARS-CoV-2 vaccine candidate.

Although we have selected MRT5500 as the lead COVID-19 vaccine candidate, and we are expecting to begin a Phase 1/2 clinical trial to evaluate MRT5500 in the first quarter of 2021, the development of a SARS-CoV-2 vaccine candidate is still in its early stages, MRT5500 may not be safe and may not successfully prevent COVID-19 in a timely manner, if at all. In order to obtain regulatory approval to market a new biological product such as a SARS-CoV-2 vaccine, we must demonstrate proof of safety, purity and potency in humans. To satisfy these requirements, we will have to conduct one or more adequate and well-controlled clinical trials. Before we can commence clinical trials for MRT5500, we must complete extensive preclinical testing and studies that support applicable regulatory approval to begin clinical trials. We cannot be certain of the timely completion or outcome of our preclinical testing and studies, and we cannot predict if any applicable regulatory bodies will accept our proposed clinical program or if the

outcome of our preclinical testing and studies will ultimately support the further development of MRT5500. As a result, we cannot be sure that we will be able to advance MRT5500 into clinical trials on the timeline we expect, if at all, and we cannot be sure that applicable regulatory authorities will allow clinical trials to begin. Moreover, even if we do initiate clinical trials for MRT5500, our development efforts may not be successful, and clinical trials that we conduct may not demonstrate sufficient safety, purity and potency necessary to obtain the requisite regulatory approvals for MRT5500.

The timing and success of any clinical trials will also depend on our ability enroll subjects in the clinical trials. Several other companies are pursuing the development of a vaccine for COVID-19, and subject enrollment may be affected by availability of a competing commercially available vaccine and other clinical trials of competing vaccine candidates. Subject enrollment may also be affected by other factors, including variations in the incidence of COVID-19 at the time of the trial and the perceived risks and benefits of the clinical trial among potential subjects. Our inability to enroll a sufficient number of subjects for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Even if we obtain positive results from preclinical studies or initial clinical trials, we may not achieve the same success in demonstrating safety and potency in later trials.

Further, while the U.S. Food and Drug Administration, or FDA, and other regulatory authorities have authority to expedite review and approval of various products, including vaccines, it is unclear how the FDA or such other authorities will exercise these powers with respect to our vaccine candidate, if at all. For example, the FDA possesses authority to issue Emergency Use Authorizations, or EUAs, allowing for the manufacture, research and distribution of drugs, biologics and medical devices that have not been approved or licensed by the agency under the standard requirements that typically govern the development of new medical products. The extent to which the FDA relies on the EUA process, or other expedited regulatory mechanisms for the study and review of vaccine candidates, remains unclear and will be subject to a number of different factors. As a result, even if our vaccine candidate shows promising results in preclinical and clinical studies, its approval under an EUA or expedited licensing processes is not assured and the need for authorization from the FDA may result in delays.

If the COVID-19 pandemic is effectively contained or the risk of SARS-CoV-2 infection is diminished or eliminated before we can successfully develop and manufacture an mRNA vaccine against SARS-CoV-2, Sanofi could de-prioritize its support for the joint development of such a vaccine. We are also committing financial resources and personnel to the development of MRT5500, which may cause delays in or otherwise negatively impact our other development programs, despite uncertainties surrounding the longevity and extent of coronavirus as a global health concern. Our business could be negatively impacted by our allocation of significant resources to a global health threat that is unpredictable and could rapidly dissipate or against which our vaccine, if developed, may not be partially or fully effective. In addition, there are numerous efforts currently underway by other parties and collaborations to develop a vaccine for COVID-19, and another party may be successful in producing a safe or efficacious vaccine or other treatment for the disease before we can complete clinical development, and, in that case, SARS-CoV-2 would be effectively contained before we are able to receive authorization for vaccine candidate.

Our failure to successfully develop, manufacture and commercialize MRT5500 could have an adverse effect on our business, prospects, financial condition and results of operations and cause us reputational harm, any of which could cause our stock price to decline.

The manufacture, scale, validation and production of a potential SARS-CoV-2 vaccine is complex and uncertain. We may encounter difficulties, and our ability to develop and provide a vaccine, if approved, could be delayed, interrupted or halted.

To date, we have produced multiple mRNA constructs and have established 100 gram single-batch production with our clinical-stage mRNA therapeutics platform. Build-out of a dedicated manufacturing space through a contract manufacturing partner was completed during the third quarter of 2020 and has the potential to accommodate multiple 250-gram batches per month upon continued investments and third-party supplier arrangements. As it relates to development of MRT5500, depending on the final human dose and timing of scale-up activities, we estimate that we could have manufacturing capacity to produce 90-360 million doses annually. However, 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 may face difficulties in production of MRT5500, including scaling up and validating production, potential shortages of raw materials, a failure by our contract manufacturer to comply with guidelines, specifications and regulations necessary for the manufacture of any vaccine candidate we seek to manufacture, and other disruptions relating to the manufacture of any potential SARS-CoV-2 vaccine candidates.

We will also require substantial capital to commence and continue production of MRT5500, which capital may not be available in the time frame or amount needed. Any delay or interruption in the supply of clinical trial supplies could delay the completion of planned clinical trials of MRT5500, increase the cost associated with the related clinical trial program 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 MRT5500 or approved vaccine may result in shipment delays, inventory shortages, lot failures, product withdrawals or recalls or other interruptions in supply.

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 losses were \$33.2 million and \$113.3 million for the nine months ended September 30, 2020 and for the year ended December 31, 2019, respectively. As of September 30, 2020, we had an accumulated deficit of \$392.7 million. We have funded our operations to date primarily through sales of equity securities and research and collaboration agreements. 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;
- continue the development of mRNA vaccine candidates against infectious diseases, including MRT5500, the lead vaccine candidate against SARS-CoV-2;
- 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 manufacturing, operational, financial and management systems;
- 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:

[Table of Contents](#)

- 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 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.

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, 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., referred to as 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 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 and cash equivalents of \$674.1 million as of September 30, 2020 will enable us to fund our operating expenses and capital expenditure requirements through 2023. 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.

Our estimates regarding our ability to fund our operating expenses and capital expenditure requirements with our existing cash and cash equivalents are based on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. Our future funding requirements will depend on, and could increase significantly as a result of, many factors, including:

- the impacts of the COVID-19 pandemic and our response to it;
- the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical studies and clinical trials;
- the success of our collaboration with Sanofi;
- the costs, timing and outcome of regulatory review of our product candidates;
- 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;
- 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;

[Table of Contents](#)

- the extent to which we acquire or in-license other product candidates and technologies; and
- the timing, receipt and amount of sales of, or milestone payments related to or royalties on, our current or future product candidates, if any.

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.

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

In December 2016, we acquired the 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 for non-CF MRT Products that are mRNA vaccines in certain Licensed Fields under the Amended Sanofi Agreement. 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.

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, 2019, we had federal net operating loss carryforwards of \$229.3 million, of which \$122.1 million will, if not utilized, begin to expire in 2031. As of December 31, 2019, we had state net operating loss carryforwards of \$210.6 million, which will, if not utilized, begin to expire in 2031. Our federal and state research and development tax credit carryforwards of \$6.5 million and \$2.7 million, respectively, will, if not utilized, begin to expire in 2032 and 2028, respectively, and orphan drug tax credit carryforwards of \$13.0 million will, if not utilized, begin to expire in 2037. We also have state investment tax credit carryforwards of \$0.3 million, which will, if not utilized, begin to expire in 2020. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset our future income tax liabilities.

In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or 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 by certain stockholders 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 Sections 382 and 383 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 an ownership change has occurred or occurs in the future 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.

There is also a risk that due to regulatory changes, such as suspensions on the use of net operating losses, or other unforeseen reasons, our existing net operating losses could expire or otherwise become unavailable to offset future income tax liabilities. As described below in “Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition,” the Tax Cuts and Jobs Act, or the Tax Act, as amended by the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, includes changes to U.S. federal tax rates and the rules governing net operating loss carryforwards that may significantly impact our ability to utilize our net operating losses to offset taxable income in the future. In addition, state net operating losses generated in one state cannot be used to offset income generated in another state. For these reasons, even if we attain profitability, we may be unable to use a material portion of our net operating losses and other tax attributes.

Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition.

Recent changes in tax law may adversely affect our business or financial condition. On December 22, 2017, the U.S. government enacted the Tax Act, which significantly reformed the Code. The Tax Act, among other things, contained significant changes to corporate taxation, including a reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, the limitation of the tax deduction for net interest expense to 30% of adjusted taxable income (except for certain small businesses), the limitation of the deduction for net operating losses arising in taxable years beginning after December 31, 2017 to 80% of current year taxable income and elimination of net operating loss carrybacks for losses arising in taxable years ending after December 31, 2017 (though any such net operating losses may be carried forward indefinitely), the allowance of immediate deductions for certain new investments instead of deductions for depreciation expense over time, and the modification or repeal of many business deductions and credits.

As part of Congress’s response to the COVID-19 pandemic, the Families First Coronavirus Response Act, or FFCR Act, was enacted on March 18, 2020, and the CARES Act was enacted on March 27, 2020. Both contain numerous tax provisions. In particular, the CARES Act retroactively and temporarily (for taxable years beginning before January 1, 2021) suspends application of the 80%-of-income limitation on the use of net operating losses, which was enacted as part of the Tax Act. It also provides that net operating losses arising in any taxable year beginning after December 31, 2017, and before January 1, 2021 are generally eligible to be carried back up to five years. The CARES Act also temporarily (for taxable years beginning in 2019 or 2020) relaxes the limitation of the tax deductibility for net interest expense by increasing the limitation from 30 to 50% of adjusted taxable income.

Regulatory guidance under the Tax Act, the FFCR Act and the CARES Act is and continues to be forthcoming, and such guidance could ultimately increase or lessen impact of these laws on our business and financial condition. It is also possible that Congress will enact additional legislation in connection with the COVID-19 pandemic, some of which could have an impact on our company. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the FFCR Act or the CARES Act.

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. For example, in September 2019 we discontinued the development of MRT5201, a liver targeted treatment for ornithine transcarbamylase, or OTC, deficiency, and terminated our Phase 1/2 clinical trial for MRT5201 in patients with OTC deficiency.

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.

If we are unable to complete satisfactorily the clinical development of, obtain marketing approval for or successfully commercialize MRT5005, 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. In April 2020, we announced that enrollment and dosing had been paused in the ongoing Phase 1/2 clinical trial in patients with CF as a consequence of the COVID-19 pandemic. In September 2020, we announced that enrollment and dosing resumed. Due to the uncertain environment associated with the COVID-19 pandemic, we are unable to predict the rate of enrollment and timing for reporting data. We expect that the COVID-19 pandemic will continue to impact our clinical trials as described above in “Risks Related to the COVID-19 Pandemic.” 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.

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

- 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;

[Table of Contents](#)

- 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.

Many of these factors are beyond our control, including clinical development, 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, 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, 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 the planned Phase 1 trial for MRT5500 and our Phase 1/2 clinical trial of MRT5005 in patients with CF, will be conducted as planned, completed on schedule, if at all, or yield positive results. For example, in April 2020, we announced that enrollment and dosing had been paused in our ongoing Phase 1/2 clinical trial in patients with CF as a consequence of the COVID-19 pandemic. In September 2020, we announced that enrollment and dosing resumed. Due to the uncertain environment associated with the COVID-19 pandemic, we are unable to predict the rate of enrollment and timing for reporting data. We expect that the COVID-19 pandemic will continue to impact our clinical trials as described above in “Risks Related to the COVID-19 Pandemic.”

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 reaching agreement on acceptable terms with 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, 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;

Table of Contents

- 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.

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 have experienced delays in enrollment and dosing in our ongoing Phase 1/2 clinical trial in patients with CF as a consequence of the COVID-19 pandemic. We could also 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, 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.

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 and MRT5500 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 mice, 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 have experienced and may in the future experience difficulty enrolling and dosing 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. We depend on Sanofi to design and conduct clinical trials for our vaccine candidates. As a result, we may not control the manner or time schedule in which these clinical trials are conducted, which may negatively impact our business operations. In addition, we anticipate competition recruiting patients for the anticipated future clinical trials of MRT5500, as many other companies are conducting or plan to conduct similar trials. In contrast, because our clinical trial of MRT5005 is 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

[Table of Contents](#)

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 April 2020, we announced that enrollment and dosing had been paused in our ongoing Phase 1/2 clinical trial in patients with CF as a consequence of the COVID-19 pandemic. Even though we were able to resume enrollment and dosing in this trial as announced in September 2020, we may encounter slower than expected enrollment or dosing delays due to the CF patient population, or CF advocacy groups may provide additional guidance for the safety of the CF population, which may delay the trial. At this time we are unable to predict the rate of enrollment and timing for reporting data. We expect that the COVID-19 pandemic will continue to impact our clinical trial of MRT5005 as described above in “Risks Related to the COVID-19 Pandemic.” 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;
- 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 the development of vaccines. However, the development of MRT5005 or MRT5500 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.

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 MRT5005, we expect to have to evaluate long-term exposure to establish the safety and tolerability of this product candidate 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 not previously 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 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.

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, MRT5500 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;

[Table of Contents](#)

- 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.

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.

[Table of Contents](#)

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, Symdeko and Trikafta, each of which is marketed by Vertex Pharmaceuticals Incorporated, or Vertex. Vertex also has several CFTR modulator compounds in clinical development, each of which is currently in a Phase 2 clinical trial.

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., Eloxix Pharmaceuticals Ltd, and Proteostasis Therapeutics, Inc.

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

Large and established companies, such as Merck & Co., Inc., GlaxoSmithKline plc, Sanofi, Pfizer, Inc., Johnson & Johnson and AstraZeneca plc, among others, compete in the vaccine market. Although there is no approved vaccine against COVID-19 on the market, competition to find a vaccine for COVID-19 is intense, and moving rapidly. We are aware of several companies, such as Moderna, Inc., Novavax, Inc., BioNTech SE and Pfizer, Inc., as well as others, that have already commenced clinical trials of a vaccine against COVID-19, and there will likely be significant and consistent competition as these active programs mature.

Risks Related to Dependence on Third Parties

We have an existing collaboration with Sanofi and we are highly dependent on the efforts of Sanofi to advance our vaccine development program, including the vaccine against SARS-CoV-2. If our collaboration with Sanofi is not successful, our business could be adversely affected.

We currently have a collaboration and license agreement with Sanofi to develop mRNA vaccines for infectious disease pathogens, including SARS-CoV-2. We have selected MRT5005 as the lead candidate for a vaccine against SARS-CoV-2. Sanofi may not be successful in its efforts to develop or commercialize our vaccine candidates, which could adversely affect our business. In addition, Sanofi could decide not to pursue or prioritize our vaccine candidates, or disputes may arise over certain obligations, which could have an adverse effect on our ability to develop and commercialize any affected product candidate.

We have limited control over the amount and timing of resources that Sanofi dedicates to the development or commercialization of our vaccine candidates. Our ability to generate revenue from our arrangement with Sanofi will depend on Sanofi's ability to successfully perform the functions assigned to Sanofi.

Our collaboration with Sanofi may pose several risks, including the following:

- Sanofi has significant discretion in determining the efforts and resources that it will apply to our collaboration;
- Sanofi may not perform its obligations as expected;
- The clinical trials conducted as part of our collaboration with Sanofi may not be successful;

[Table of Contents](#)

- Sanofi may not pursue development and/or commercialization of any vaccine candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in Sanofi's strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- Sanofi has final decision-making authority for conducting clinical trials, and this may result in Sanofi delaying clinical trials, providing insufficient funding for clinical trials, stopping a clinical trial or abandoning a vaccine candidate, repeating or conducting new clinical trials or requiring a new formulation of a vaccine candidate for clinical testing;
- We may not have access to, or may be restricted from disclosing, certain information regarding vaccine candidates being developed or commercialized under our collaboration with Sanofi and, consequently, may have limited ability to inform our stockholders about the status of such vaccine candidates;
- Sanofi has an existing collaboration with GlaxoSmithKline plc to develop a SARS-CoV-2 vaccine candidate that could compete with MRT5500, and Sanofi could further independently develop, or develop with third parties, products that compete directly or indirectly with any of our vaccine candidates if Sanofi believes that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- Sanofi may view vaccine candidates developed in collaboration with us as competitive with their own product candidates or products, which may cause Sanofi to cease to devote resources to the commercialization of our vaccine candidates;
- Sanofi may not commit sufficient resources to the marketing and distribution of any such of our vaccine candidates that achieve regulatory approval;
- Disagreements with Sanofi, including disagreements over proprietary rights, contract interpretation or the preferred course of development of any of our vaccine candidates, may cause delays or termination of the research, development, manufacture or commercialization of such vaccine candidates, may lead to additional responsibilities for us with respect to such vaccine 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 our contractual obligations to Sanofi and any upstream contractual obligations we may owe to our licensors or other third parties;
- Sanofi 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 collaboration with Sanofi;
- Sanofi may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- Sanofi may terminate our collaboration for convenience after a specified notice period and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable vaccine candidates.

[Table of Contents](#)

If our collaboration with Sanofi does not result in the successful development and commercialization of vaccines, or if Sanofi 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 our agreement with Sanofi, our development of vaccine candidates could be delayed and we may need additional resources to develop our vaccine candidates.

In addition, if Sanofi 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 Sanofi's activities.

We may enter into additional collaborations with third parties, and if we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

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. Our likely collaborators for any other collaboration arrangements include large and mid-size pharmaceutical companies and biotechnology companies.

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. Even if we are able to successfully enter into collaborations with third parties for one or more of our programs or product candidates, such collaborations may be subject to risks similar to those described above under the risk factor captioned "We have an existing collaboration with Sanofi and we are highly dependent on the efforts of Sanofi to advance our vaccine development program, including the vaccine against SARS-CoV-2. If our collaboration with Sanofi is not successful, our business could be adversely affected."

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.

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 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. In addition, as a consequence of the COVID-19 pandemic, some of these parties may be unable to perform their engagements satisfactorily or at all. 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. We expect that the COVID-19 pandemic will continue to impact our clinical trials as described above in "Risks Related to the COVID-19 Pandemic."

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, 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, as a consequence of the COVID-19 pandemic, our third-party manufacturers may experience delays or other interruptions with their manufacturing capabilities and may be unable to perform satisfactorily, 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 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. For example, we are party to a leasing arrangement with a third-party manufacturer, Albany Molecular Research, Inc., or AMRI, for the manufacture of certain portions of our product candidates. Although we were closely involved with the design and construction of the cleanroom suites, we may still experience delays in the development services provided by AMRI. Such delays could materially adversely affect our business.

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.

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, 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, including vaccines, 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, including vaccines, 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.

[Table of Contents](#)

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 and vaccines 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.

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.

[Table of Contents](#)

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;
- 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

Unfavorable U.S. or global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the U.S. and global economy and financial markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including, weakened demand for our products, if any, and could adversely impact our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for any current or future approved products. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business. We expect that the COVID-19 pandemic will continue to disrupt the financial markets and may adversely impact economies worldwide as described above in “Risks Related to the COVID-19 Pandemic.”

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;
- 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.

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 that protect our technology or product candidates or that effectively prevent others from commercializing competitive technologies and product candidates being issued. 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.

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 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.

[Table of Contents](#)

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 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.

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 November 2, 2020, six of our patents issued in Europe are under opposition, including two 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.

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 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 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

[Table of Contents](#)

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. Further, as set forth above in the risk factor captioned "We and Sanofi may not be successful in our joint efforts to successfully develop in an expedited timeframe an mRNA vaccine against SARS-CoV-2, the virus responsible for COVID-19," substantial uncertainty and potential delay surrounds the use of emergency authorization processes to approve products, including vaccines, by the FDA and comparable regulatory authorities in other jurisdictions. The extent to which the FDA and other regulatory authorities rely on such emergency procedures is uncertain and subject to a number of different factors. As a result, even if the FDA and comparable regulatory authorities have indicated that they will rely on these emergency procedures to review our product candidates, including our vaccine candidates, approval of such products under these procedures may be substantially delayed and is not assured.

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. We may also experience delays as a consequence of the COVID-19 pandemic. 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.

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. Following protracted negotiations, the United Kingdom left the EU on January 31, 2020. Under the withdrawal agreement, there is a transitional period until December 31, 2020 (extendable up to two years). Discussions between the United Kingdom and the EU have so far mainly focused on finalizing withdrawal issues and transition agreements but have been extremely difficult to date. To date, only an outline of a trade agreement has been reached. Much remains open but the Prime Minister has indicated that the United Kingdom will not seek to extend the transitional period beyond the end of 2020. If no trade agreement has been reached before the end of the transitional period, there may be significant market and economic disruption.

Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety, and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales, and distribution of pharmaceutical products is derived from EU directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, may force us 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.

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. We may seek orphan drug designations for MRT5005 for other indications or for other of our product candidates. There can be no assurances that we will be able to obtain such designations.

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, 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.

Although we have received fast track designation for MRT5005 for CF, such designation may not actually lead to a faster development or regulatory review or approval process. In addition, we may not receive such designation for other product candidates.

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. We have received fast track designation for MRT5005 for CF. However, even though we received 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. In addition, if we seek fast track designation for other product candidates, we may not receive it from the FDA.

The FDA has granted a Rare Pediatric Disease Designation, or RPDD, for MRT5005 and we may seek the same designation for one or more of our other product candidates. However, a BLA for one or more of our product candidates may not meet the eligibility criteria for a priority review voucher upon approval nor does this designation mean that our product candidates, including MRT5005, will be subject to a faster development and regulatory review process.

With enactment of the Food and Drug Administration Safety and Innovation Act in 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications that meet the criteria specified in the law. This provision is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application.

For the purposes of this program, a "rare pediatric disease" is a (a) serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and (b) rare disease or conditions within the meaning of the Orphan Drug Act. In March 2020, the FDA granted Rare Pediatric Designation for MRT5005 for the treatment of CF. The FDA may, however, determine that a BLA for this product candidate or our other product candidates do not meet the eligibility criteria for a priority review voucher upon approval. Moreover, even if one or more of our product candidates does satisfy those criteria, the product will need to be licensed before September 30, 2022, in order to be granted a rare disease priority review voucher.

While the opportunity to receive a priority review voucher was meant to expire for those companies that had not received a designation by September 30, 2020, Congress authorized a short-term extension on that date. According to these new sunset provisions, after December 11, 2020, the FDA may only award a voucher for an approved RPDD product application if the sponsor has the designation for the drug and that designation was granted by December 11, 2020. After December 11, 2022, the FDA may not award any priority review vouchers. The Creating Hope Reauthorization Act, which was received in the Senate on September 30, 2020, proposes to replace those cutoffs with September 30, 2024 and September 30, 2026, respectively, thus extending the authorized period for RPDD and granting of RPDD priority review vouchers from the 21st Century Cures Act by four years. It remains to be seen, however, whether such legislation will be enacted into law.

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.

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.

[Table of Contents](#)

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;
- 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;

[Table of Contents](#)

- 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
- *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.

Compliance with global privacy and data security requirements could result in additional costs and liabilities to us or inhibit our ability to collect and process data globally, and the failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the EU General Data Protection Regulation, or the GDPR, which took effect across all member states of the European Economic Area, or EEA, in May 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 increases our obligations with respect to clinical trials conducted in the EEA by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States, and, as a result, increases the scrutiny that clinical trial sites located in the EEA should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the United States. The GDPR also permits data protection authorities to require destruction of improperly gathered or used personal information and or impose substantial fines for violations of the GDPR, which can be up to four percent of global revenues or 20 million Euros, whichever is greater and it 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. In addition, the GDPR provides that European Union member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data.

Similar actions are either in place or under way in the United States. There are a broad variety of data protection laws that are applicable to our activities, and a wide range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General all are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered at both the state and federal levels. For example, the California Consumer Privacy Act—which went into effect on January 1, 2020—is creating similar risks and obligations as those created by GDPR, though the Act does exempt certain information collected as part of a clinical trial subject to the Federal Policy for the Protection of Human Subjects (the Common Rule). Many other states are considering similar legislation. A broad range of legislative measures also have been introduced at the federal level. Accordingly, failure to comply with federal and state laws (both those currently in effect and future legislation) regarding privacy and security of personal information could expose us to fines and penalties under such laws. There also is the threat of consumer class actions related to these laws and the overall protection of personal data. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with these requirements is rigorous and time intensive and requires significant resources and a review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data collected in the European Union. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of

sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations.

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 2029. The CARES Act suspended the 2% Medicare sequester from May 1, 2020 through December 31, 2020, and extended the sequester by one year, through 2030. The American Taxpayer Relief Act of 2012 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, and continue to be, 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 President Trump 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, will become effective in 2019. Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the ACA-mandated “Cadillac” tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. Further, the Bipartisan Budget Act of 2018, among other things, amended the ACA, effective January 1, 2019, to increase from 50 to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole.” The Congress may consider other legislation to replace elements of the ACA during the next Congressional session.

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. On April 28, 2020, that decision was reversed by the U.S. Supreme Court.

In addition, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the ACA is an essential and inseparable feature of the ACA, and therefore because the mandate was repealed as part of the Tax Cuts and Jobs Act, the remaining provisions of the ACA are invalid as well. The Trump administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the same judge issued an order staying the judgment pending appeal. The Trump Administration recently represented to the Court of Appeals considering this judgment that it does not oppose the lower court’s ruling. On July 10, 2019, the Court of Appeals for the Fifth Circuit heard oral argument in this case. On December 18, 2019, that court affirmed the lower court’s ruling that the individual mandate portion of the ACA is unconstitutional and it remanded the case to the district court for reconsideration of the severability question and additional analysis of the provisions of the ACA. On January 21, 2020, the U.S. Supreme Court declined to review this decision on an expedited basis. On March 3, 2020, the Court agreed to hear the case through its normal procedures. On June 25, 2020, the Trump Administration and a coalition of 18 states asked the court to strike down the entirety of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

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 proposals contain further drug price control measures that could be enacted during the 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, or HHS, 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. Finally, the current presidential administration's budget proposal for fiscal year 2021 includes a \$135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower-cost generic and biosimilar drugs.

In addition, on July 24, 2020, President Trump issued four executive orders intended to lower the costs of prescription drug products. The first order would require all federally qualified health centers to pass on to patients the discounts the health centers receive on insulin and epinephrine through Medicare's 340B Drug Discount Program. The second order would establish an international pricing index that would set the price Medicare Part B pays for the costliest medications covered under the program to the lowest price in other economically advanced countries.

The third order was intended to reduce the costs of drugs by supporting the safe importation of prescription drugs. Specifically, the order calls upon the HHS to facilitate grants to individuals of waivers of the prohibition of importation of prescription drugs that would allow patients to import FDA approved drug products from abroad, so long as doing so would result in lower costs. In addition, the order would allow wholesalers and pharmacies to re-import both biological drugs and insulin that were originally manufactured in the United States and then exported for international sale. This action preceded the finalization of a rulemaking on September 24, 2020 that allows states or certain other non-federal government entities to submit importation program proposals to the FDA for review and approval. Applicants are required to demonstrate that their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. The FDA has issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products).

The fourth order would end drug rebates used by health plan sponsors, pharmacies or pharmacy benefit managers, or PBMs, in operating the Medicare Part D program. Specifically, the order directs HHS to exclude from safe harbor protections under the federal anti-kickback statute retroactive price reductions that are not applied at the point-of-sale. Instead, the order requires HHS to establish new safe harbors that would allow health plan sponsors, pharmacies, and PBMs to pass on those discounts to consumers at point-of-sale in order to lower the patient's out-of-pocket costs and permit the use of certain bona fide PBM service fees. Each of these orders directs the federal government to implement the initiatives outlined in the orders, meaning they will not have immediate effects.

Following issuance of these orders, President Trump issued a fifth executive order which instructs the federal government to develop a list of "essential" medicines and then buy them and other medical supplies from U.S. manufacturers instead of from companies around the world, including especially China. The order is meant reduce regulatory barriers to domestic pharmaceutical manufacturing and catalyze manufacturing technologies needed to keep drug prices low and the production of drug products in the United States.

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 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 and vaccines, if any.

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals and vaccines 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 or vaccine. 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 or vaccine to other available therapies. If reimbursement of our drugs or vaccines 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. We also have one stockholder who holds approximately 24% 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 July 2020, we issued and sold 4,884,434 shares of common stock to the Investor in connection with the Second Sanofi Amendment. We registered these shares on a registration statement in August 2020, covering the resale by the Investor of the 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, or the Securities Act. The Investor will not, and will cause its affiliates not to, sell or transfer the shares without our prior written approval, subject to specified conditions, until January 2022. 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

[Table of Contents](#)

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. Furthermore, we have registered an aggregate of 14,042,632 shares of our common stock on Form S-8 to be issued to our employees under certain employee benefit 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 an indeterminate amount of 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. We are party to an Open Market Sale AgreementSM, or Sales Agreement, with Jefferies LLC, or Jefferies, pursuant to which, from time to time, we may offer and sell through Jefferies up to \$100.0 million of common stock pursuant to one or more “at the market” offerings. Through September 30, 2020, pursuant to the Sales Agreement, we had issued and sold an aggregate of 2,863,163 shares of our common stock, for gross proceeds of \$37.9 million, before deducting commissions of \$1.1 million and other offering expenses of \$0.2 million. There were no shares issued or sold pursuant to the Sales Agreement during the three months ended September 30, 2020. In June 2020, we completed a public offering in which we issued and sold 5,681,819 shares of common stock and a stockholder sold 6,824,992 shares of common stock registered under our universal shelf registration statement. 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 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;
- public announcements about the scientific community’s evolving understanding of the COVID-19 pandemic and the potential effectiveness of vaccines, treatments, public health measures and other approaches to addressing the disease;
- 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;

Table of Contents

- 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, such as the impact of the COVID-19 pandemic on our industry and market conditions; and
- the other factors described in this “Risk Factors” section.

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.

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 cease to be an emerging growth company, or EGC, as of December 31, 2020, 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. In addition, after we cease to be an EGC, we will be required to comply with auditor attestation requirements, increased disclosure obligations and other reporting requirements, which will likely increase our costs in the upcoming fiscal year. 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.

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 us 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. These choice of forum provisions will not apply to suits brought to enforce a duty or liability created by the Securities Act, the Exchange Act of 1934, as amended, or any other claim for which federal courts have exclusive jurisdiction. 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

[Table of Contents](#)

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.

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 5. Other Information.

On November 3, 2020, referred to as the Lease Commencement Date, we entered into an Indenture of Lease, or the Lease, with Boston Properties Limited Partnership, a Delaware limited partnership, referred to as the Landlord, for approximately 138,444 square feet of office and laboratory space, or the Premises, for our new headquarters to be located at 200 West Street, Waltham, Massachusetts 02451. The Lease has an initial 10-year term, and we have an option to extend the Lease for one additional 10-year term.

We are obligated to pay rent beginning 12 months after the Lease Commencement Date, referred to as the Rent Commencement Date, subject to a possible 180-day extension due to COVID-19 pandemic-related work delays. Base rent shall be \$5.7 million for the first year following the Rent Commencement Date and approximately \$8.0 million for the second year following the Rent Commencement Date and thereafter shall be subject to a 3% annual increase. Our aggregate minimum annual lease payment under the Lease during the initial term shall be \$86.8 million. We also will be required to pay certain operating expenses of the Premises, including our share of real estate taxes and utilities.

Landlord has provided us with an allowance, or the TI Allowance, for tenant improvements of \$190.00 per square foot of the Premises. The TI Allowance can be used for payment of design, permits, and construction costs in connection with the construction of such tenant improvements. The TI Allowance shall be paid by the Landlord as a reimbursement so long as we have satisfied the specified requisition conditions, including the completion of the tenant improvements.

During the first seven years of the initial 10-year term of the Lease, we may elect to expand the Premises to include the entire rentable floor area of a new wing to be constructed in a location adjacent to the Premises, which is currently anticipated to contain 130,000 square feet of rentable floor area, referred to as the Expansion Space. Base rent for the Expansion Space, if we elect to lease such space, shall be the amount equal to an annual rate of return on the total project costs (as reasonably determined by the Landlord) equal to 275 basis points over the average yield on 10-year U.S. Treasury notes. If we elect to expand the Premises, we and the Landlord will negotiate in good faith an amendment to the Lease, which shall include terms such as commencement date, rent commencement date and any tenant improvement allowance for the Expansion Space.

We shall have the right to sublet or assign the Lease, subject to the Landlord's consent. In the event we sublet more than 50% of the Premises for all or substantially all of the remaining lease term, then the Landlord shall have the right to terminate the Lease. The Landlord also has the right to terminate the Lease upon default under the Lease by us, such default including, but not limited to, failure to pay rent, an assignment or sublet in violation of the Lease, our bankruptcy or insolvency, and abandonment of the Premises.

Table of Contents

Item 6. Exhibits.

| <u>Exhibit Number</u> | <u>Description</u> |
|-----------------------|---|
| 10.1*+ | First Amendment, dated September 22, 2020 to Suite Retention and Development Agreement, dated September 9, 2019, by and between Albany Molecular Research, Inc. and the Registrant. |
| 31.1* | 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. |
| 31.2* | 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. |
| 32.1** | 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. |
| 32.2** | Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. |
| 101.INS* | Inline XBRL Instance Document– the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document |
| 101.SCH* | Inline XBRL Taxonomy Extension Schema Document |
| 101.CAL* | Inline XBRL Taxonomy Extension Calculation Linkbase Document |
| 101.DEF* | Inline XBRL Taxonomy Extension Definition Linkbase Document |
| 101.LAB* | Inline XBRL Taxonomy Extension Label Linkbase Document |
| 101.PRE* | Inline XBRL Taxonomy Extension Presentation Linkbase Document |
| 104 | Cover Page Interactive Data File (formatted as Inline XBRL with applicable taxonomy extension information contained in Exhibits 101) |

* Filed herewith.

** Furnished herewith.

+ Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed. Double asterisks denote omissions.

**AMENDMENT #1
TO SUITE RETENTION AND DEVELOPMENT AGREEMENT**

THIS AMENDMENT #1 (the “Amendment”) to the SUITE RETENTION AND DEVELOPMENT AGREEMENT dated September 9, 2019 (the “Agreement”) is made and entered into as of September 22, 2020 (the “Amendment Effective Date”), by and between Albany Molecular Research, Inc. with a place of business at 26 Corporate Circle, Albany, New York 12203 (“AMRI”) and Translate Bio, Inc. with a place of business at 29 Hartwell Avenue, Lexington, Massachusetts 02421 (“Translate Bio”). For purposes of this Amendment, AMRI and Translate Bio are each a “Party” and collectively, the “Parties.”

WHEREAS, Translate Bio has requested that AMRI use the Cleanroom Suites to perform additional services relating to the manufacture of Translate Bio’s bulk drug product, and AMRI has agreed to perform such additional services;

NOW, THEREFORE, in consideration of the mutual covenants contained herein and other valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties hereby agree as follows:

ARTICLE I

AMENDMENTS

1.1 Article I of the Agreement is hereby amended to add the following definitions (in alphabetical order, as applicable):

- (a) “Build-Out Completion” means August 31, 2020.
- (b) “Bulk Drug Product” means the bulk drug product after Processing by AMRI in accordance with the Master Batch Record. For the avoidance of doubt, unless expressly agreed by the Parties in writing the Processing of Bulk Drug Product pursuant to this Agreement shall in no event include Secondary Packaging.
- (c) “Bulk Drug Product-Specific Program Technology” means any Program Technology that constitutes an improvement, modification, derivative, or new use of the Raw Materials proprietary to Translate Bio or a Translate Bio partner or of the Drug Product, including without limitation the manufacturing process for Drug Product as set forth in the Master Batch Record, but which is not an improvement of general applicability to the manufacturing process, and all intellectual property rights in any of the foregoing.

- (d) “Bulk Drug Product Specifications” means the written specifications and quality standards, including tests, analytical procedures and acceptance criteria, that are established to confirm the characteristics and quality of the Bulk Drug Product as set forth in the mutually agreed upon Master Batch Record, and as amended from time to time, by written agreement of the Parties when applicable, in accordance with the procedures set forth in this Agreement and the Quality Agreement.
- (e) “Packaging” of Bulk Drug Product means the filling of vials or other receptacles, as applicable, and bulk packaging of naked vials or other receptacles, as applicable, into containers. For the avoidance of doubt, this shall exclude vial labeling and Secondary Packaging.
- (f) “Process,” “Processed,” or “Processing” means (i) as relates to Drug Substance, the conversion of the Raw Materials into Drug Substance in accordance with the Master Batch Record and the terms and conditions set forth in this Agreement and the Quality Agreement, and (ii) as relates to Bulk Drug Product, the compounding, processing, formulating and/or Packaging of the Drug Substance and Raw Materials into Bulk Drug Product in accordance with the Master Batch Record and the terms and conditions set forth in this Agreement and the Quality Agreement.
- (g) “Secondary Packaging” means all packaging components or packaging activities not specifically included under the definition for Packaging, which activities are not and will not be in direct contact with the dosage form. (For the avoidance of doubt, Secondary Packaging shall include the finished labelling of vials containing the Bulk Drug Product).
- (h) “Translate Bio Materials” means certain Raw Materials and any other materials (such as consumables) which Translate Bio shall supply to AMRI, as mutually agreed by the Parties. Translate Bio Materials shall include Drug Substance which is Delivered by AMRI to Translate Bio (as defined in Section 5.1 herein) or which is manufactured by a third party and provided to AMRI by or on behalf of Translate Bio.
- (i) “Translate Bio Technology” means Translate Bio Existing Technology, Drug-Substance-Specific Program Technology and Bulk Drug Product-Specific Program Technology.

1.2 Article I of the Agreement is hereby amended to delete the definition of Drug Substance Warranty.

1.3 The first section of Section 2.1 is hereby replaced in its entirety with the following:

In connection with this Agreement, AMRI and Translate Bio shall arrange for the design and construction of a series of cleanroom suites and warehouse space at the Facility, as more specifically described in Exhibit A (the “Cleanroom Suites”). Title to, and risk of loss of, the Cleanroom Suites shall be retained by AMRI. AMRI shall

enter into the Detailed Design Contract and the Construction Contract, pursuant to which the design and construction, respectively, of the Cleanroom Suites (the "Build-Out") shall be conducted in conformance with Translate Bio's requirements (as mutually agreed by the Parties) and the terms of and conditions of this Agreement. The Parties agree that the Build-Out, and specifically design of the Cleanroom Suites, shall be a joint effort between the Parties who shall share responsibility in ensuring that the Cleanroom Suites satisfy Translate Bio's objectives. For clarity, the rights of Translate Bio set forth in Section 2.7 of the Agreement shall apply with respect to the warehouse space funded by the Translate Bio Capital Investment, provided that AMRI shall be able to use unoccupied ambient space within the warehouse for temporary storage of consumables unrelated to mRNA manufacture, but shall make such space available as needed for Translate Bio purposes.

1.4 Section 2.8(d) is hereby replaced in its entirety with the following:

(i) Monthly Payment. On a monthly basis during the Term, beginning with the month immediately following the Build-Out Completion, Translate Bio shall have an obligation to pay AMRI an amount equal to (a) one million U.S. Dollars (\$1,000,000) (as may be increased pursuant to the immediately following paragraph, the "Translate Bio Monthly Fee"), plus (b) the Incremental Monthly Fee (as defined in Section 3.2), if any (the Translate Bio Monthly Fee and the Incremental Monthly Fee, collectively, the "Aggregate Monthly Payment"). Translate Bio shall owe a pro-rated payment for the month during which the Build-Out Completion occurs, equivalent to: the Translate Bio Monthly Fee (i) divided by the number of days in such month, and (ii) multiplied by the number of days remaining in such month following the day of the month that Build-Out Completion occurred (inclusive of such day). The Parties agree that the Translate Bio Monthly Fee shall be made to AMRI in exchange for the Suite Retention and the Development Services (excluding any Batches covered by the Incremental Monthly Fee and any services expressly set forth in Exhibit D, which shall be subject to the charges set forth therein). Notwithstanding the foregoing, the Parties agree that Translate Bio shall be obligated to pay the Translate Bio Monthly Fee, irrespective of whether AMRI is providing Development Services during the relevant monthly period. Furthermore, the Translate Bio Monthly Fee is not refundable or transferrable, and is not creditable to any future services or periods covered by this Agreement.

AMRI may implement an increase in the Translate Bio Monthly Fee once annually, beginning on January 1 of each calendar year following the first anniversary of Build-Out Completion, in an amount equal to three percent (3%) of the then-current Translate Bio Monthly Fee. AMRI shall provide Translate Bio with written notice of any such increase at least [**] prior to its effective date.

(ii) Invoicing and Payments.

(a) Translate Bio Monthly Fee. AMRI shall provide an invoice to Translate Bio for the Translate Bio Monthly Fee [**] prior to the first day of the applicable month, and Translate Bio shall make payment of such Translate Bio Monthly Fee on the first Business Day of such month.

- (b) Incremental Monthly Fee. AMRI shall provide an invoice to Translate Bio for the Incremental Monthly Fee, if any, as soon as possible after the number of Batches of Drug Substance and/or Bulk Drug Product that AMRI completed during such month are known, which shall in no case be earlier than the end of such month. Translate Bio shall make payment of such Incremental Monthly Fee within [**] of receipt of such invoice.

(iii) Forecast and Purchase Orders.

- (a) Forecast. On the first day of each calendar month, beginning approximately [**] prior to the date of Build-Out Completion, Translate Bio shall furnish to AMRI a written [**] rolling forecast of quantities of Drug Substance and/or Bulk Drug Product Batches that Translate Bio intends to order from AMRI during such period.
- (b) Purchase Orders. For purchase orders relating to the Translate Bio Monthly Fee, upon Build-Out Completion, Translate Bio shall provide a purchase order to AMRI for Translate Bio Monthly Fees for the period from Build-Out Completion (pro-rated for the month in which Build-Out Completion occurs) through the end of the calendar year. Thereafter on an annual basis, [**] prior to the start of each calendar year, Translate Bio shall provide a purchase order to AMRI for Translate Bio Monthly Fees for the following calendar year (January 1 through December 31).

1.5 The third sentence of Section 3.1 is hereby replaced in its entirety with the following:

Translate Bio is obligated to pay for (via the Translate Bio Monthly Fee, the Incremental Monthly Fee and any applicable additional costs per Section 2.8(e)), and AMRI shall have no liability for, all Development Services even if the Drug Substance and/or Bulk Drug Product produced does not conform to the Drug Substance Specifications or Bulk Drug Product Specifications, respectively, unless such non-conformity is attributable to AMRI's gross negligence or willful misconduct.

1.6 Section 3.2 is hereby replaced in its entirety with the following:

Monthly Batches and Pricing. As set forth in Section 2.8(d)(iii)(a), Translate Bio shall specify the number of Batches of Drug Substance and/or Bulk Drug Product to be Processed by AMRI in any given calendar month. Pricing for such Batches shall be as follows (as may be increased pursuant to this Section 3.2, the "Batch Pricing"):

| | |
|----------------------------|--------|
| Batch of Drug Substance | \$[**] |
| Batch of Bulk Drug Product | \$[**] |

The Batch Pricing set forth in the table above assumes: (i) operation of the Cleanroom Suites for no more than eight hours per day, five days per week, (ii) that each Batch of Drug Substance shall take [**] to Process and each Batch of Bulk Drug Product shall

take [**] to Process, and (iii) Batch size of Drug Substance equal to [**], and Batch size of Drug Product equal to the liposomal encapsulation of one Batch of Drug Substance. AMRI shall only be obligated to Process the number of Batches of Drug Substance and/or Bulk Drug Product permitted by such one-shift operations during each month. The Parties agree that, [**] from the Amendment Effective Date, the Parties will negotiate in good faith any increase or decrease in the Batch Pricing in the event that the actual Processing time for a Batch of Drug Substance and/or a Batch of Bulk Drug Product is longer or shorter than the estimates set forth above.

AMRI may implement an increase in the Batch Pricing once annually, beginning on January 1 of each calendar year following the first anniversary of Build-Out Completion, in an amount equal to three percent (3%) of the then-current Batch Pricing. AMRI shall provide Translate Bio with written notice of any such increase at least [**] prior to its effective date.

On a monthly basis, Translate Bio shall owe to AMRI the greater of (i) the Translate Bio Monthly Fee, or (ii) the aggregate amount due for all Batches of Drug Substance and Bulk Drug Product completed during such month (any amount over \$1,000,000 shall be referred to as the "Incremental Monthly Fee"). For purposes of this provision, completion of a Batch shall mean that AMRI has fulfilled its Processing obligations (which, for the avoidance of doubt, shall not include testing or release).

By way of example:

- (a) If AMRI completes Processing of [**] of Drug Substance and [**] of Bulk Drug Product in a month, Translate Bio shall owe the Translate Bio Monthly Fee [**].
- (b) If AMRI completes Processing of [**] of Drug Substance and [**] of Bulk Drug Product in a month, Translate Bio shall owe the Translate Bio Monthly Fee [**].

1.7 Section 3.3 is hereby replaced in its entirety with the following:

Rescheduling and Cancellation. In the event that Translate Bio, with less than [**] notice prior to the scheduled date of manufacture, cancels, or delays or reschedules by more than [**], any Batches for a month in which there is an Incremental Monthly Fee (based on the forecast provided in Section 2.8(d)(iii)(a)), Translate Bio shall pay an amount equal to [**] percent ([**]%) of the applicable Incremental Monthly Fee. By way of example:

- (a) if AMRI is scheduled to Process [**] of Drug Substance and [**] of Bulk Drug Product in a month and Translate Bio cancels or reschedules [**] of Drug Substance, Translate shall owe \$[**].

- (b) if AMRI is scheduled to Process [**] of Drug Substance and [**] of Bulk Drug Product in a month and Translate Bio cancels or reschedules [**] of Bulk Drug Product, Translate shall owe \$[**].

For the avoidance of doubt, this Section 3.3 shall in no event limit Translate Bio's obligation to pay the Translate Bio Monthly Fee each month.

- 1.8 The first sentence of Section 4.1 of the Agreement is hereby replaced in its entirety with the following:

Translate Bio shall supply to AMRI for Processing, at Translate Bio's sole cost, the Translate Bio Materials.

- 1.9 Article 5 of the Agreement is hereby replaced in its entirety with the following:

5.1 Delivery of Drug Substance and/or Bulk Drug Product. Title and risk of loss of Drug Substance, Bulk Drug Product and other deliverables shall transfer from AMRI to Translate Bio upon delivery EXW Facility (Incoterms 2010) ("Delivery"). For manufactured Batches of Bulk Drug Product, Delivery shall occur upon the later of (a) the issuance to Translate Bio of the completed Batch Documentation, in accordance with the Quality Agreement and (b) notification by AMRI to Translate Bio that the Batch is available for pick up at Facility. For manufactured Batches of Drug Substance, Delivery shall occur upon the issuance to Translate Bio of the completed Batch Documentation, in accordance with the Quality Agreement. Translate Bio is responsible for transportation of the Bulk Drug Product to Translate Bio's final destination, at the sole risk and expense of Translate Bio. In the event that Translate Bio elects for Drug Substance Processed by AMRI not be used to Process Bulk Drug Product pursuant to this Agreement, Translate Bio is responsible for transportation of the Drug Substance to Translate Bio's final destination, at the sole risk and expense of Translate Bio. For avoidance of doubt, in each case, Translate Bio is responsible for arranging pick up by carrier and all shipping costs and risks. Should Translate Bio request AMRI to assist with any arrangements with the carrier, such arrangements will be made by AMRI on behalf of Translate Bio in accordance with Translate Bio's applicable instructions and at the sole risk and expense of Translate Bio.

5.2 Storage.

(a) Bulk Drug Product. If Translate Bio does not pick up Bulk Drug Product after AMRI has notified Translate Bio in writing that it is available, AMRI shall store such Bulk Drug Product at the Facility. For all Bulk Drug Product stored by AMRI following Delivery, Translate Bio agrees that: (i) Translate Bio has title and risk of ownership, (ii) Translate Bio has made a fixed commitment to purchase such Bulk Drug Product, (iii) Translate Bio is responsible for any decrease in market value of such Bulk Drug Product that relates to factors and circumstances outside of AMRI's control, and (iv) Translate Bio is responsible for obtaining insurance for such Bulk Drug Product during the storage period, if desired.

(b) Drug Substance. For all Drug Substance that has been Processed by AMRI and which has not yet been used for Processing of Bulk Drug Product or which Translate Bio has elected not be used to Process Bulk Drug Product pursuant to this Agreement, AMRI shall store such Drug Substance at the Facility. For all Drug Substance stored by AMRI following Delivery, Translate Bio agrees that: (i) Translate Bio has title and risk of ownership, (ii) Translate Bio has made a fixed commitment to purchase such Drug Substance, (iii) Translate Bio is responsible for any decrease in market value of such Drug Substance that relates to factors and circumstances outside of AMRI's control, and (iv) Translate Bio is responsible for obtaining insurance for such Drug Substance during the storage period, if desired.

(c) Storage fees. The storage fees outlined in Exhibit D do not apply if storage of Drug Substance or Bulk Drug Product is within the warehouse space indicated as part of the "Cleanroom Suite" referenced in Exhibit A. For clarity, the Parties agree that such storage fees shall apply to storage of Drug Substance and/or Bulk Drug Product in the event that the warehouse space indicated as part of the Cleanroom Suite is full.

1.10 Section 7.1(c) is hereby replaced in its entirety with the following:

(c) all Drug Substance and/or Bulk Drug Product delivered hereunder shall be manufactured in accordance with cGMP, the then-current Master Batch Record and the Quality Agreement, and, if manufactured after establishment of a validated manufacturing process, shall conform to the Drug Substance Specifications and/or Bulk Drug Product Specifications, as applicable, upon Delivery;

1.11 Section 10.1 is hereby replaced in its entirety with the following:

Indemnification by AMRI. AMRI shall defend (upon Translate Bio's written request), indemnify and hold harmless Translate Bio, its Affiliates, and their respective directors, officers, employees and agents ("Translate Bio Indemnitees") from and against any and all suits, claims, losses, demands, liabilities, damages, costs and expenses (including reasonable attorneys' fees) resulting from or arising out of any suit, demand or action by any third party ("Losses") to the extent (i) caused by the gross negligence or willful misconduct of any AMRI Indemnitee; (ii) arising from a claim by a third party that AMRI Technology used in the performance of the Development Services or Process (on its own and not in combination with any Translate Bio Materials or Translate Bio Technology) infringes such third party's intellectual property right; or (iii) caused by the breach of any of AMRI's representations, warranties (including without limitation the representations made in Section 7.1(c) herein) or obligations under this Agreement, in each case relating to the Development Services only; except to the extent of the amount of any Losses arising out of claims for which Translate Bio is obligated to indemnify AMRI hereunder.

1.12 The second sentence of Section 8.1 is hereby amended in its entirety as follows:

Confidential Information of Translate Bio includes, but is not limited to Translate Bio Existing Technology, Drug Substance-Specific Program Technology, Bulk Drug Product-Specific Program Technology, the Master Batch Record, the Drug Substance Specifications and the Bulk Drug Product Specifications.

1.13 The first sentence of Section 9.3 is hereby amended in its entirety as follows:

All Drug Substance-Specific Program Technology and Bulk Drug Product-Specific Program Technology shall be the exclusive property of Translate Bio, and AMRI shall and hereby does assign all of its rights, title and interest in and to the Drug Substance-Specific Program Technology and the Bulk Drug Product-Specific Program Technology to Translate Bio, and shall take such actions as are reasonably requested by Translate Bio, at Translate Bio's expense, to effect the foregoing assignment and in connection with Translate Bio's efforts to secure patent protection for such Drug Substance-Specific Program Technology and Bulk Drug Product-Specific Program Technology.

1.14 Except for those provisions of the Agreement referenced in Sections 1.3 through 1.13 above (which shall be as expressly set forth above), all references in the Agreement to "Drug Substance" shall become references to "Drug Substance and/or Bulk Drug Product, as applicable" and all references to "Drug Substance Specifications" shall become references to "Drug Substance Specifications and/or Bulk Drug Product Specifications, as applicable."

1.15 The Parties expressly agree that the references to Drug Substance in the Agreement, solely as relates to its use in the Processing of Bulk Drug Product, shall include (i) Drug Substance Processed by AMRI pursuant to the Agreement, or (ii) drug substance which is otherwise manufactured by or on behalf of Translate Bio and provided to AMRI (the "Third Party Drug Substance"). For the avoidance of doubt, Translate Bio's obligations with respect to Translate Bio Materials, as set forth in Section 4.1 of the Agreement, shall apply to any Third Party Drug Substance. The Parties further agree that AMRI's obligations with respect to any testing of the Third Party Drug Substance shall only be as expressly agreed by the Parties in writing. Notwithstanding the foregoing, AMRI shall not be responsible for any defects in the Third Party Drug Substance.

1.16 In Exhibit B, the table labeled "Translate Bio Equipment" is hereby replaced in its entirety with the table labeled with same name in Exhibit B-1 attached hereto.

1.17 The Parties agree that promptly following the Amendment Effective Date, they will prepare a matrix which sets forth the raw materials and consumables to be provided by each of Translate Bio and AMRI with respect to both the Drug Substance and the Bulk Drug Product (provided that Drug Substance shall constitute Translate Bio materials, even if not included on such matrix). Once both Parties have approved in writing, such

matrix shall be binding provided that the Parties may mutually agree to change the allocation of procurement responsibilities between the Parties for such materials in writing [**] period following the Amendment Effective Date. For clarity, in the event new materials are required with respect to the Processing of Drug Substance and/or Bulk Drug Product or any new products are added to this Agreement, those changes will be managed through the appropriate quality and operational systems (e.g., tech transfer, change control) on a timely basis as required for the Parties to perform their responsibilities pursuant to this Agreement.

1.18 Article 14 is hereby amended as follows:

The "To AMRI" notice is amended and restated as follows:

Albany Molecular Research, Inc.
26 Corporate Circle
Albany, New York 12212
Attn: Steve Lichter

ARTICLE II
MISCELLANEOUS

- 2.1 All capitalized terms used but not defined herein shall have the meaning set forth in the Agreement.
- 2.2 Except as expressly set forth herein, the amendments provided herein shall not, by implication or otherwise, limit, constitute a waiver of, or otherwise affect the rights and remedies of AMRI or Translate Bio under the Agreement, nor shall it constitute a waiver of any default, nor shall it alter, modify, amend or in any way affect any of the terms, conditions, obligations, covenants or agreements contained in the Agreement. The amendments provided herein shall apply and be effective only with respect to the provisions of the Agreement specifically referred to by such amendments and all other terms and conditions in the Agreement shall remain in full force and effect and are hereby ratified and confirmed.
- 2.3 This Amendment may be executed in two (2) or more counterparts, each of which will be deemed an original, but all of which together will constitute one (1) and the same instrument. Any photocopy, PDF or electronic reproduction of the executed Amendment shall constitute an original.

IN WITNESS WHEREOF, the Parties hereto have executed this Amendment as of the Amendment Effective Date.

ALBANY MOLECULAR RESEARCH, INC.

By: /s/ Steve Lichter

Name: Steve Lichter

Title: President

TRANSLATE BIO, INC.

By: /s/ Paul Burgess

Name: Paul Burgess

Title: COO and CLO

**CERTIFICATION 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**

I, Ronald C. Renaud, Jr., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Translate Bio, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 5, 2020

By: _____ /s/ Ronald C. Renaud, Jr.

Ronald C. Renaud, Jr.
President and Chief Executive Officer

**CERTIFICATION 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**

I, John R. Schroer, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Translate Bio, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 5, 2020

By: _____ /s/ John R. Schroer

John R. Schroer
Treasurer and Chief Financial 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 September 30, 2020 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: November 5, 2020

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 September 30, 2020 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: November 5, 2020

By: _____ /s/ John R. Schroer

John R. Schroer
Treasurer and Chief Financial Officer