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**UNITED STATES**

**SECURITIES AND EXCHANGE COMMISSION**

**Washington, D.C. 20549**

**\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_**

**FORM 10-Q**

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|  |  |  |  |  |  |
| ☒ | | | **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 No. 001-35890**

\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

**Millendo Therapeutics, Inc.**

(Exact Name of Registrant as Specified in its Charter)

**\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_**

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| **Delaware** | | |  | | | **45-1472564** | | |
| **(State or Other Jurisdiction of**  **Incorporation or Organization)** | | |  | | | **(I.R.S. Employer**  **Identification No.)** | | |
|  | | |  | | |  | | |
| **110 Miller Avenue, Suite 100**  **Ann Arbor, Michigan** | | |  | | | **48104** | | |
| **(Address of Principal Executive Offices)** | | |  | | | **(Zip Code)** | | |

**Registrant’s telephone number, including area code: (734) 845-9000**

(Former Name, Former Address and Former Fiscal Year, if Changed Since Last Report)

\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

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

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |
| **Title of each class** | | | **Trading Symbol(s)** | | | **Name of each exchange on which registered** | | |
| Common Stock, $0.001 par value | | | MLND | | | The Nasdaq Global Market | | |

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

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

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

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

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

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

The number of shares of Registrant’s Common Stock, $0.001 par value per share, outstanding as of November 3, 2020 was 18,999,701.

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**SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS**

Unless the context suggests otherwise, references in this Quarterly Report on Form 10-Q to “Millendo,” “the Company,” “we,” “us,” and “our” refer to Millendo Therapeutics, Inc. and, where appropriate, its subsidiaries.

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These forward-looking statements reflect our plans, estimates and beliefs and include, but are not limited to, statements about our plans to develop and commercialize our product candidates; the initiation, timing, progress and results of current and future clinical trials of MLE-301, a selective neurokinin 3 receptor (NK3R) antagonist, for the treatment of vasomotor symptoms (“VMS”) in menopausal women, including our Phase 1 clinical trial of MLE-301, the impact of our discontinuation of the development of livoletide as a potential treatment of patients with Prader-Willi syndrome (“PWS”); the impact of ceasing the investment in the development of nevanimibe as a potential treatment for classic congenital adrenal hyperplasia (“CAH”); the timing of and our ability to obtain and maintain regulatory approvals for our product candidates; the impact of the COVID-19 pandemic on our business, preclinical studies and clinical development programs and timelines, our financial condition and results of operations; and our estimates regarding future revenue, if any, future expenses, the funding of our operations, including whether our existing cash, cash equivalents and restricted cash will be sufficient to fund our current operating plans into 2022, as well as our future capital requirements and needs for additional financing. These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. In some cases, you can identify forward-looking statements by terms such as “anticipates,” “believes,” “could,” “estimates,” “expects,” “intends,” “may,” “plans,” “potential,” “predicts,” “projects,” “should,” “would,” and similar expressions intended to identify forward-looking statements. Forward-looking statements reflect our current views with respect to future events and are based on assumptions and subject to risks and uncertainties. Because of these risks and uncertainties, the forward-looking events and circumstances discussed in this report may not transpire. These risks and uncertainties include, but are not limited to, the risks included in this Quarterly Report on Form 10-Q under Part II, Item 1A, “Risk Factors.” A summary of selected risks associated with our business are set forth below.

Given these uncertainties, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent our estimates and assumptions only as of the date of this document. You should read this document with the understanding that our actual future results may be materially different from what we expect. Except as required by law, we do not undertake any obligation to update or revise any forward-looking statements contained in this report, whether as a result of new information, future events or otherwise.

**SUMMARY OF SELECTED RISKS ASSOCIATED WITH OUR BUSINESS**

Our business is subject to numerous risks and uncertainties, including those discussed at length in the section titled "Risk Factors." These risks include, among others, the following:

•We have incurred significant operating losses since inception and anticipate that we will continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.

•We have a limited operating history and have never generated any revenue from product sales, which may make it difficult to assess our future viability.

•We will require additional capital to finance our operations, which may not be available on acceptable terms, if at all. Failure to obtain capital when needed may force us to delay, limit or terminate certain of our development programs, future commercialization efforts or other operations.

•Raising additional capital by issuing equity or debt securities may cause dilution to our existing stockholders, and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights.

•We may be required to make payments under licenses applicable to nevanimibe and MLE-301.

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

•Our future success is dependent on the successful clinical development, regulatory approval and subsequent commercialization of MLE-301 and any future product candidates. If we are not able to obtain the required regulatory approvals, we will not be able to commercialize our current or future product candidates and our ability to generate revenue will be adversely affected.

•Preclinical studies or earlier clinical trials are not necessarily predictive of future results and the results of our clinical trials may not support our MLE-301 claims.

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•We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

•If we are not able to obtain required regulatory approvals, we will not be able to commercialize MLE-301 or any future product candidate and our ability to generate revenue will be harmed.

•If we are unable to establish sales, marketing and distribution capabilities, either on our own or in collaboration with third-parties, we may not be successful in commercializing our product candidates, if approved.

•Even if we obtain and maintain approval for our current and future product candidates from the FDA, we may nevertheless be unable to obtain approval for our product candidates outside of the United States, which would limit our market opportunities and could harm our business.

•If we are not able to obtain orphan drug designations or exclusivity for any of our current or future product candidates for which we seek such designation, the potential profitability of any such product candidates could be limited.

•We rely on the availability of licenses for intellectual property from third-parties and these licenses may not be available to us on commercially reasonable terms, or at all.

•If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

•We jointly own patents and patent applications with third-parties. Our ability to exploit or enforce these patent rights, or to prevent the third-party from granting licenses to others with respect to these patent rights, may be limited in some circumstances

•We do not have our own manufacturing capabilities and will rely on third-parties to produce clinical and commercial supplies of MLE-301 and any future product candidates.

•We rely on third-parties to conduct, supervise and monitor our clinical trials, and if those third-parties perform in an unsatisfactory manner, it may harm our business.

•Our business, preclinical studies and clinical development programs and timelines, our financial condition and results of operations could be materially and adversely affected by the current COVID-19 pandemic.

•We are highly dependent on the services of our key executives and personnel, including Julia C. Owens, Ph.D., our chief executive officer, Christophe Arbet-Engels, M.D., Ph.D., our chief medical officer, and Ryan Zeidan, Ph.D., our chief development officer, and if we are not able to retain these members of our management team or recruit and retain additional management, clinical and scientific personnel, our business will be harmed.

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**PART I – FINANCIAL INFORMATION**

**Item 1 – Financial Statements**

**MILLENDO THERAPEUTICS, INC.**

**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 | | | $ | 43,212 |  |  | | | $ | 62,478 |  |
| Short-term restricted cash | | | 540 | |  |  | | | 1,034 | |  |
|  | | |  | | |  | | |  | | |
| Prepaid expenses and other current assets | | | 2,093 | |  |  | | | 6,344 | |  |
| Refundable tax credit | | | 244 | |  |  | | | 1,276 | |  |
| Total current assets | | | 46,089 | |  |  | | | 71,132 | |  |
|  | | |  | | |  | | |  | | |
| Operating lease right-of-use assets | | | 2,360 | |  |  | | | 3,331 | |  |
| Other assets | | | 389 | |  |  | | | 507 | |  |
| Total assets | | | $ | 48,838 |  |  | | | $ | 74,970 |  |
| **Liabilities and stockholders’ equity** | | |  | | |  | | |  | | |
| Current liabilities: | | |  | | |  | | |  | | |
| Current portion of debt | | | $ | 222 |  |  | | | $ | 208 |  |
| Accounts payable | | | 1,733 | |  |  | | | 1,495 | |  |
| Accrued expenses | | | 4,267 | |  |  | | | 9,066 | |  |
| Operating lease liabilities — current | | | 899 | |  |  | | | 1,751 | |  |
| Total current liabilities | | | 7,121 | |  |  | | | 12,520 | |  |
| Debt, net of current portion | | | 117 | |  |  | | | 168 | |  |
| Operating lease liabilities | | | 1,786 | |  |  | | | 2,395 | |  |
| Other liabilities | | | — | |  |  | | | 16 | |  |
| Total liabilities | | | 9,024 | |  |  | | | 15,099 | |  |
| Commitments and contingencies (Note 6) | | |  | | |  | | |  | | |
| Stockholders’ equity: | | |  | | |  | | |  | | |
| Preferred stock, $0.001 par value: 5,000,000 shares authorized; no shares issued and outstanding | | | — | |  |  | | | — | |  |
| Common stock, $0.001 par value: 100,000,000 shares authorized; 18,999,701 and 18,266,545 shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively | | | 19 | |  |  | | | 18 | |  |
| Additional paid-in capital | | | 276,551 | |  |  | | | 267,018 | |  |
| Accumulated deficit | | | (237,698) | |  |  | | | (208,654) | |  |
| Accumulated other comprehensive income | | | 274 | |  |  | | | 165 | |  |
| Total stockholders’ equity attributable to Millendo Therapeutics, Inc. | | | 39,146 | |  |  | | | 58,547 | |  |
| Equity attributable to noncontrolling interests | | | 668 | |  |  | | | 1,324 | |  |
| Total stockholders’ equity | | | 39,814 | |  |  | | | 59,871 | |  |
| Total liabilities and stockholders’ equity | | | $ | 48,838 |  |  | | | $ | 74,970 |  |

See accompanying Notes to unaudited Interim Consolidated Financial Statements

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**MILLENDO THERAPEUTICS, INC.**

**Consolidated Statements of Operations and Comprehensive Loss**

**(Unaudited)**

**(in thousands except share and per share amounts)**

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Three Months Ended September 30,** | | | | | | | | |  | | | **Nine Months Ended September 30,** | | | | | | | | |
|  | | | **2020** | | |  | | | **2019** | | |  | | | **2020** | | |  | | | **2019** | | |
| **Operating expenses:** | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Research and development | | | $ | 2,676 |  |  | | | $ | 7,308 |  |  | | | $ | 16,682 |  |  | | | $ | 19,493 |  |
| General and administrative | | | 3,380 | |  |  | | | 4,443 | |  |  | | | 12,113 | |  |  | | | 13,075 | |  |
| Loss from operations | | | 6,056 | |  |  | | | 11,751 | |  |  | | | 28,795 | |  |  | | | 32,568 | |  |
| **Other expenses (income):** | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Interest expense (income), net | | | $ | 8 |  |  | | | $ | (238) |  |  | | | $ | (159) |  |  | | | $ | (866) |  |
| Other loss | | | 310 | |  |  | | | 119 | |  |  | | | 408 | |  |  | | | 167 | |  |
| Net loss | | | $ | (6,374) |  |  | | | $ | (11,632) |  |  | | | $ | (29,044) |  |  | | | $ | (31,869) |  |
|  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
|  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Net loss per share of common stock, basic and diluted | | | $ | (0.34) |  |  | | | $ | (0.87) |  |  | | | $ | (1.54) |  |  | | | $ | (2.38) |  |
| Weighted-average shares of common stock outstanding, basic and diluted | | | 18,999,701 | |  |  | | | 13,420,614 | |  |  | | | 18,816,481 | |  |  | | | 13,386,381 | |  |
| Other comprehensive income (loss): | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Foreign currency translation adjustment | | | $ | 156 |  |  | | | $ | (17) |  |  | | | $ | 109 |  |  | | | $ | (25) |  |
| Comprehensive loss | | | $ | (6,218) |  |  | | | $ | (11,649) |  |  | | | $ | (28,935) |  |  | | | $ | (31,894) |  |
|  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
|  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |

See accompanying Notes to unaudited Interim Consolidated Financial Statements

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**MILLENDO THERAPEUTICS, INC.**

**Consolidated Statements of Stockholders’ Equity (Deficit)**

**(Unaudited)**

**(in thousands except share amounts)**

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Three Months Ended September 30, 2020** | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
|  | | | **Common Stock** | | | | | | | | |  | | | **Additional Paid-in Capital** | | |  | | | **Accumulated Deficit** | | |  | | | **Accumulated Other Comprehensive Income** | | |  | | | **Total Stockholders’ Equity (Deficit) attributable to Millendo Therapeutics, Inc.** | | |  | | | **Total Equity Attributable to Noncontrolling Interests** | | |  | | | **Total Stockholders’ Equity (Deficit)** | | |
|  | | | **Shares** | | |  | | | **Amount** | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Balance at July 1, 2020 | | | 18,999,701 | |  |  | | | $ | 19 |  |  | | | $ | 275,463 |  |  | | | $ | (231,324) |  |  | | | $ | 118 |  |  | | | $ | 44,276 |  |  | | | $ | 668 |  |  | | | $ | 44,944 |  |
|  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
|  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
|  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Stock-based compensation expense | | | — | |  |  | | | — | |  |  | | | 1,088 | |  |  | | | — | |  |  | | | — | |  |  | | | 1,088 | |  |  | | | — | |  |  | | | 1,088 | |  |
| Foreign currency translation adjustment | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | 156 | |  |  | | | 156 | |  |  | | | — | |  |  | | | 156 | |  |
| Net loss | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | (6,374) | |  |  | | | — | |  |  | | | (6,374) | |  |  | | | — | |  |  | | | (6,374) | |  |
| Balance at September 30, 2020 | | | 18,999,701 | |  |  | | | $ | 19 |  |  | | | $ | 276,551 |  |  | | | $ | (237,698) |  |  | | | $ | 274 |  |  | | | $ | 39,146 |  |  | | | $ | 668 |  |  | | | $ | 39,814 |  |

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Nine Months Ended September 30, 2020** | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
|  | | | **Common Stock** | | | | | | | | |  | | | **Additional Paid-in Capital** | | |  | | | **Accumulated Deficit** | | |  | | | **Accumulated Other Comprehensive Income** | | |  | | | **Total Stockholders’ Equity (Deficit) attributable to Millendo Therapeutics, Inc.** | | |  | | | **Total Equity Attributable to Noncontrolling Interests** | | |  | | | **Total Stockholders’ Equity (Deficit)** | | |
|  | | | **Shares** | | |  | | | **Amount** | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Balance at January 1, 2020 | | | 18,266,545 | |  |  | | | $ | 18 |  |  | | | $ | 267,018 |  |  | | | $ | (208,654) |  |  | | | $ | 165 |  |  | | | $ | 58,547 |  |  | | | $ | 1,324 |  |  | | | $ | 59,871 |  |
| Issuance of common stock, net of issuance costs | | | 719,400 | |  |  | | | 1 | |  |  | | | 5,649 | |  |  | | | — | |  |  | | | — | |  |  | | | 5,650 | |  |  | | | — | |  |  | | | 5,650 | |  |
| Exercise of stock options | | | 1,449 | |  |  | | | — | |  |  | | | 2 | |  |  | | | — | |  |  | | | — | |  |  | | | 2 | |  |  | | | — | |  |  | | | 2 | |  |
| Exercise/forfeiture of BSPCE warrants | | | 12,307 | |  |  | | | — | |  |  | | | 734 | |  |  | | | — | |  |  | | | — | |  |  | | | 734 | |  |  | | | (656) | |  |  | | | 78 | |  |
| Stock-based compensation expense | | | — | |  |  | | | — | |  |  | | | 3,148 | |  |  | | | — | |  |  | | | — | |  |  | | | 3,148 | |  |  | | | — | |  |  | | | 3,148 | |  |
| Foreign currency translation adjustment | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | 109 | |  |  | | | 109 | |  |  | | | — | |  |  | | | 109 | |  |
| Net loss | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | (29,044) | |  |  | | | — | |  |  | | | (29,044) | |  |  | | | — | |  |  | | | (29,044) | |  |
| Balance at September 30, 2020 | | | 18,999,701 | |  |  | | | $ | 19 |  |  | | | $ | 276,551 |  |  | | | $ | (237,698) |  |  | | | $ | 274 |  |  | | | $ | 39,146 |  |  | | | $ | 668 |  |  | | | $ | 39,814 |  |

See accompanying Notes to unaudited Interim Consolidated Financial Statements

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**MILLENDO THERAPEUTICS, INC.**

**Consolidated Statements of Stockholders’ Equity (Deficit)**

**(Unaudited)**

**(in thousands except share amounts)**

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Three Months Ended September 30, 2019** | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
|  | | | **Common Stock** | | | | | | | | |  | | | **Additional Paid-in Capital** | | |  | | | **Accumulated Deficit** | | |  | | | **Accumulated Other Comprehensive Income** | | |  | | | **Total Stockholders’ Equity (Deficit) attributable to Millendo Therapeutics, Inc.** | | |  | | | **Total Equity Attributable to Noncontrolling Interests** | | |  | | | **Total Stockholders’ Equity (Deficit)** | | |
|  | | | **Shares** | | |  | | | **Amount** | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Balance at July 1, 2019 | | | 13,412,058 | |  |  | | | $ | 13 |  |  | | | $ | 237,136 |  |  | | | $ | (184,323) |  |  | | | $ | 140 |  |  | | | $ | 52,966 |  |  | | | $ | 2,059 |  |  | | | $ | 55,025 |  |
| Exercise of stock options | | | 51,278 | |  |  | | | — | |  |  | | | 194 | |  |  | | | — | |  |  | | | — | |  |  | | | 194 | |  |  | | | — | |  |  | | | 194 | |  |
| Exercise/forfeiture of BSPCE warrants | | | 9,601 | |  |  | | | — | |  |  | | | 367 | |  |  | | | — | |  |  | | | — | |  |  | | | 367 | |  |  | | | (304) | |  |  | | | 63 | |  |
| Stock-based compensation expense | | | — | |  |  | | | — | |  |  | | | 1,196 | |  |  | | | — | |  |  | | | — | |  |  | | | 1,196 | |  |  | | | — | |  |  | | | 1,196 | |  |
| Foreign currency translation adjustment | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | (17) | |  |  | | | (17) | |  |  | | | — | |  |  | | | (17) | |  |
| Net loss | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | (11,632) | |  |  | | | — | |  |  | | | (11,632) | |  |  | | | — | |  |  | | | (11,632) | |  |
| Balance at September 30, 2019 | | | 13,472,937 | |  |  | | | $ | 13 |  |  | | | $ | 238,893 |  |  | | | $ | (195,955) |  |  | | | $ | 123 |  |  | | | $ | 43,074 |  |  | | | $ | 1,755 |  |  | | | $ | 44,829 |  |

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Nine Months Ended September 30, 2019** | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
|  | | | **Common Stock** | | | | | | | | |  | | | **Additional Paid-in Capital** | | |  | | | **Accumulated Deficit** | | |  | | | **Accumulated Other Comprehensive Income** | | |  | | | **Total Stockholders’ Equity (Deficit) attributable to Millendo Therapeutics, Inc.** | | |  | | | **Total Equity Attributable to Noncontrolling Interests** | | |  | | | **Total Stockholders’ Equity (Deficit)** | | |
|  | | | **Shares** | | |  | | | **Amount** | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Balance at January 1, 2019 | | | 13,357,999 | |  |  | | | $ | 13 |  |  | | | $ | 234,876 |  |  | | | $ | (164,086) |  |  | | | $ | 148 |  |  | | | $ | 70,951 |  |  | | | $ | 2,171 |  |  | | | $ | 73,122 |  |
| Exercise of stock options | | | 97,225 | |  |  | | | — | |  |  | | | 360 | |  |  | | | — | |  |  | | | — | |  |  | | | 360 | |  |  | | | — | |  |  | | | 360 | |  |
| Exercise/forfeiture of BSPCE warrants | | | 17,713 | |  |  | | | — | |  |  | | | 527 | |  |  | | | — | |  |  | | | — | |  |  | | | 527 | |  |  | | | (416) | |  |  | | | 111 | |  |
| Stock-based compensation expense | | | — | |  |  | | | — | |  |  | | | 3,130 | |  |  | | | — | |  |  | | | — | |  |  | | | 3,130 | |  |  | | | — | |  |  | | | 3,130 | |  |
| Foreign currency translation adjustment | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | (25) | |  |  | | | (25) | |  |  | | | — | |  |  | | | (25) | |  |
| Net loss | | | — | |  |  | | | — | |  |  | | | — | |  |  | | | (31,869) | |  |  | | | — | |  |  | | | (31,869) | |  |  | | | — | |  |  | | | (31,869) | |  |
| Balance at September 30, 2019 | | | 13,472,937 | |  |  | | | $ | 13 |  |  | | | $ | 238,893 |  |  | | | $ | (195,955) |  |  | | | $ | 123 |  |  | | | $ | 43,074 |  |  | | | $ | 1,755 |  |  | | | $ | 44,829 |  |

See accompanying Notes to unaudited Interim Consolidated Financial Statements

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**MILLENDO THERAPEUTICS, INC.**

**Consolidated Statements of Cash Flows**

**(Unaudited)**

**(in thousands)**

|  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Nine Months Ended September 30,** | | | | | | | | |
|  | | | **2020** | | |  | | | **2019** | | |
| **Operating activities:** | | |  | | |  | | |  | | |
| Net loss | | | $ | (29,044) |  |  | | | $ | (31,869) |  |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |  | | |  | | |  | | |
| Depreciation | | | 115 | |  |  | | | 52 | |  |
| Stock-based compensation expense | | | 3,148 | |  |  | | | 3,130 | |  |
| Foreign currency remeasurement loss | | | 395 | |  |  | | | — | |  |
|  | | |  | | |  | | |  | | |
|  | | |  | | |  | | |  | | |
| Amortization of right-of-use asset | | | 748 | |  |  | | | 719 | |  |
| Loss on disposal of equipment | | | 6 | |  |  | | | — | |  |
|  | | |  | | |  | | |  | | |
| Changes in operating assets and liabilities: | | |  | | |  | | |  | | |
| Prepaid expenses and other current assets | | | 5,243 | |  |  | | | (505) | |  |
| Other assets | | | 21 | |  |  | | | 175 | |  |
| Accounts payable | | | 280 | |  |  | | | 812 | |  |
| Accrued expenses and other liabilities | | | (4,805) | |  |  | | | (1,204) | |  |
| Operating lease liabilities | | | (1,239) | |  |  | | | (841) | |  |
| Cash used in operating activities | | | (25,132) | |  |  | | | (29,531) | |  |
| **Investing activities:** | | |  | | |  | | |  | | |
| Purchase of property and equipment | | | (26) | |  |  | | | (364) | |  |
| Proceeds from sale of marketable securities | | | — | |  |  | | | 4,385 | |  |
|  | | |  | | |  | | |  | | |
| Cash (used in) provided by investing activities | | | (26) | |  |  | | | 4,021 | |  |
| **Financing activities:** | | |  | | |  | | |  | | |
| Repayment of debt | | | (53) | |  |  | | | (134) | |  |
| Payment of financing costs | | | (197) | |  |  | | | (245) | |  |
| Proceeds from the issuance of common stock, net of issuance costs | | | 5,650 | |  |  | | | — | |  |
|  | | |  | | |  | | |  | | |
|  | | |  | | |  | | |  | | |
| Proceeds from option and BSPCE warrant exercises | | | 78 | |  |  | | | 471 | |  |
| Repayment of principal on finance lease | | | (28) | |  |  | | | (9) | |  |
| Cash provided by financing activities | | | 5,450 | |  |  | | | 83 | |  |
| Effect of foreign currency exchange rate changes on cash | | | (52) | |  |  | | | 5 | |  |
| Net decrease in cash, cash equivalents and restricted cash | | | (19,760) | |  |  | | | (25,422) | |  |
| Cash, cash equivalents and restricted cash at beginning of period | | | 63,512 | |  |  | | | 73,770 | |  |
| Cash, cash equivalents and restricted cash at end of period | | | $ | 43,752 |  |  | | | $ | 48,348 |  |
| **Supplemental schedule of non-cash investing and financing activities:** | | |  | | |  | | |  | | |
| Right-of-use assets acquired under operating leases | | | $ | — |  |  | | | $ | 3,414 |  |
|  | | |  | | |  | | |  | | |

See accompanying Notes to unaudited Interim Consolidated Financial Statements

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**MILLENDO THERAPEUTICS, INC.**

**Notes to Unaudited Interim Consolidated Financial Statements**

**1. Organization and Description of Business**

***Description of Business***

Millendo Therapeutics, Inc. (the “Company”), a Delaware corporation, together with its subsidiaries, is a clinical stage biopharmaceutical company primarily focused on developing novel treatments for endocrine diseases where current therapies do not exist or are insufficient. The Company seeks to leverage its understanding of recent biological discoveries in endocrinology to continue to advance and build its pipeline in order to improve the lives of patients.

The Company has a selective neurokinin 3 receptor (NK3R) antagonist (MLE-301) in its research and development pipeline, which it is developing as a potential treatment of vasomotor symptoms (“VMS”), commonly known as hot flashes and night sweats, in menopausal women. The Company is also actively pursuing additional pipeline assets in treatment areas where it has knowledge and experience in developing drug product candidates. The Company seeks to identify assets that complement its current portfolio.

The Company had been developing livoletide (AZP-531) as a potential treatment for Prader-Willi syndrome (“PWS”), a rare and complex genetic endocrine disease characterized by hyperphagia, or insatiable hunger. The Company discontinued the development of livoletide as a potential treatment for PWS in April 2020 based upon results from its Phase 2b trial. All costs, including estimated closeout costs associated with the livoletide program were recognized during the second quarter, which resulted in the Company recording $3.1 million in the second quarter of 2020. The Company recorded additional expense in the third quarter of 2020 related to the livoletide program, which reflects changes to estimated closeout costs. The Company does not expect to incur future material expenses related to this program.

In an effort to streamline costs after discontinuing the PWS program, the Company eliminated employee positions representing approximately 30% of its prior headcount, which were completed in the second quarter of 2020. The Company recorded one-time costs of $1.1 million in the form of termination benefits related to this plan in the second quarter of 2020.

The Company had also been developing nevanimibe (ATR-101) as a potential treatment for patients with classic congenital adrenal hyperplasia, (“CAH”), a rare, monogenic adrenal disease that requires lifelong treatment with exogenous cortisol, often at high doses. The Company elected to cease investing in the development of nevanimibe as a potential treatment for CAH in June 2020 based on an interim review of data from its Phase 2b trial. All costs, including estimated closeout costs associated with the nevanimibe program for the treatment of CAH were recognized during the second quarter of 2020. The Company recorded additional expense in the third quarter of 2020 related to the nevanimibe program, which reflects changes to estimated close out costs. The Company does not expect to incur future material expenses related to this program.

The Company’s operations to date have focused on conducting preclinical studies and clinical trials, acquiring technology and assets, organization and staffing, business planning, and raising capital. The Company does not have any products approved for sale and has not generated any revenue from product sales. The Company’s product candidate is subject to long development cycles and the Company may be unsuccessful in its efforts to develop, obtain regulatory approval for or market its product candidate.

The Company is subject to a number of risks including, but not limited to, the need to obtain adequate additional funding for the ongoing and planned clinical development of its current or future product candidates. Because of the numerous risks and uncertainties associated with pharmaceutical products and development, the Company is unable to accurately predict the timing or amount of funds required to complete development of its current or future product candidates, and costs could exceed the Company’s expectations for a number of reasons, including reasons beyond the Company’s control.

***Liquidity***

The Company has incurred net losses since inception and it expects to generate losses from operations for the foreseeable future primarily due to research and development costs for its potential product candidate. As of September 30, 2020, the Company had cash, cash equivalents and restricted cash of $43.8 million and an accumulated deficit of $237.7 million.

In December 2019, the Company sold a total of 4,791,667 shares of its common stock pursuant to an underwriting agreement (the “Underwriting Agreement”) with Citigroup Global Markets Inc. and SVB Leerink LLC, as representatives of the several underwriters named herein (the “Underwriters”), for total net proceeds of approximately $26.5 million, after deducting

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underwriting discounts and commissions and other offering expenses payable by the Company. The price to the public in this offering was $6.00 per share and resulted in the sale of 4,166,667 shares of the Company's common stock for net proceeds of approximately $23.0 million, after deducting underwriting discounts and commissions and other offering expenses payable by the Company. In addition, the Underwriters purchased an additional 625,000 shares of the Company's common stock at the public offering price of $6.00 per share pursuant to a purchase option granted to them under the Underwriting Agreement, resulting in net proceeds of approximately $3.5 million, after deducting underwriting discounts and commissions.

In April 2019, the Company entered into an “at-the-market” (“ATM”) equity distribution agreement with Citigroup Global Markets Inc. acting as sole agent with an aggregate offering value of up to $50.0 million, which allows the Company to sell its common stock through the facilities of the Nasdaq Capital Market. Subject to the terms of the ATM equity distribution agreement, the Company is able to determine, at its sole discretion, the timing and number of shares to be sold under this ATM facility. In March 2020, the Company amended and restated the equity distribution agreement to include SVB Leerink LLC as an additional sales agent for the ATM. In March 2020, the Company sold 719,400 shares of its common stock under its ATM equity distribution agreement for net proceeds of approximately $5.7 million.

The Company will require additional capital in the future through equity or debt financings, partnerships, collaborations, or other sources to carry out the Company’s planned development activities and to obtain regulatory approval for or to commercialize its product candidate. If additional capital is not secured when required, the Company may need to delay or curtail its operations until such funding is received. Various internal and external factors will affect whether and when the Company’s product candidate become an approved drug. The regulatory approval and market acceptance of the Company’s proposed future product (if any), length of time and cost of developing and commercializing the product candidate and/or failure of it at any stage of the drug approval process will materially affect the Company’s financial condition and future operations. The Company believes its cash, cash equivalents and restricted cash at September 30, 2020 are sufficient to fund its current operations for at least 12 months following the issuance of these financial statements.

**2. Basis of Presentation and Summary of Significant Accounting Policies**

***Basis of presentation and consolidation principles***

The accompanying unaudited Interim Consolidated Financial Statements include the accounts of Millendo Therapeutics, Inc. and its subsidiaries, and all intercompany amounts have been eliminated. The unaudited Interim Consolidated Financial Statements have been prepared in conformity with U.S. generally accepted accounting principles (“GAAP”). Any reference in these notes to applicable guidance is meant to refer to GAAP as found in the Accounting Standards Codification (“ASC”) and Accounting Standards Updates (“ASU”) of the Financial Accounting Standards Board (“FASB”). The unaudited Interim Consolidated Financial Statements include the accounts of the Company’s subsidiaries in which the Company holds a controlling financial interest as of the financial statement date.

***Unaudited Interim Consolidated Financial Statements***

The Company has prepared the accompanying unaudited Interim Consolidated Financial Statements based on Securities and Exchange Commission (“SEC”) rules that permit reduced disclosure for interim periods. These unaudited Interim Consolidated Financial Statements include, in the Company’s opinion, all adjustments, consisting only of normal recurring adjustments that the Company considers necessary for a fair presentation of its consolidated financial position and results of operations for these periods. The Company’s historical results are not necessarily indicative of the results to be expected in the future and the Company’s operating results for the three and nine months ended September 30, 2020 are not necessarily indicative of the results that may be expected for the year ending December 31, 2020.

The accompanying unaudited Interim Consolidated Financial Statements should be read in conjunction with the Consolidated Financial Statements and notes thereto included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2019 as filed with the SEC on March 11, 2020. Since the date of such financial statements, there have been no changes to the Company’s significant accounting policies except as noted below:

***Use of estimates***

The preparation of the Consolidated Financial Statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and reported amounts of expenses during the reporting period. Due to the uncertainty of factors surrounding the estimates or judgments used in the preparation of the Consolidated Financial Statements, actual results may

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materially vary from these estimates. Estimates and assumptions are periodically reviewed and the effects of revisions are reflected in the financial statements in the period they are determined to be necessary.

The Company anticipates that the COVID-19 pandemic will have an impact on clinical and preclinical development activities. Estimates and assumptions about future events and their effects cannot be determined with certainty and therefore require the exercise of judgment. As of the date of issuance of these financial statements, the Company is not aware of any specific event or circumstance that would require the Company to update its estimates, assumptions and judgments or revise the carrying value of its assets or liabilities. These estimates may change as new events occur and additional information is obtained and are recognized in the consolidated financial statements as soon as they become known. Actual results could differ from those estimates and any such differences may be material to the Company’s financial statements.

***Significant Risks and Uncertainties***

With the global spread of the ongoing COVID-19 pandemic in 2020, the Company has implemented business continuity plans designed to address and mitigate the impact of the COVID-19 pandemic on its business. The Company anticipates that the COVID-19 pandemic will continue to have an impact on clinical and preclinical development activities. The extent to which the COVID-19 pandemic impacts the Company’s business, its preclinical and clinical development and regulatory efforts, its corporate development objectives and the value of and market for its common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the U.S., Europe and other countries, and the effectiveness of actions taken globally to contain and treat the disease. The global economic slowdown, the overall disruption of global healthcare systems and the other risks and uncertainties associated with the pandemic could have a material adverse effect on the Company’s business, financial condition, results of operations and growth prospects.

In addition, the Company is subject to other challenges and risks specific to its business and its ability to execute on its strategy, as well as risks and uncertainties common to companies in the pharmaceutical industry with development operations, including, without limitation, risks and uncertainties associated with: obtaining regulatory approval of its product candidates, loss of single source suppliers or failure to comply with manufacturing regulations, identifying, acquiring or in-licensing additional products or product candidates; pharmaceutical product development and the inherent uncertainty of clinical success; and the challenges of protecting and enhancing its intellectual property rights; complying with applicable regulatory requirements. In addition, to the extent the ongoing COVID-19 pandemic adversely affects its business and results of operations, the Company may also have the effect of heightening many of the other risks and uncertainties discussed above.

***Net loss per share***

Basic loss per share of common stock is computed by dividing net loss attributable to common stockholders by the weighted-average number of shares of common stock outstanding during each period. Diluted loss per share of common stock includes the effect, if any, from the potential exercise or conversion of securities, such as restricted stock and stock options, which would result in the issuance of incremental shares of common stock. In computing the basic and diluted net loss per share, the weighted-average number of shares of common stock remains the same for both calculations due to the fact that when a net loss exists, dilutive shares are not included in the calculation as the impact is anti-dilutive.

The following potentially dilutive securities have been excluded from the computation of diluted weighted-average shares of common stock outstanding, as they would be anti-dilutive (amounts shown as common stock equivalents):

|  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **September 30,** | | | | | | | | |
|  | | | **2020** | | |  | | | **2019** | | |
| Stock options | | | 3,754,176 | |  |  | | | 2,588,235 | |  |
|  | | |  | | |  | | |  | | |
| Common stock warrants | | | 17,125 | |  |  | | | 17,125 | |  |
|  | | |  | | |  | | |  | | |
| BSA and BSPCE warrants | | | 48,265 | |  |  | | | 126,699 | |  |
|  | | | 3,819,566 | |  |  | | | 2,732,059 | |  |

***Recent accounting pronouncements***

In January 2020, the FASB issued ASU 2020-01, *Investments-Equity Securities (Topic 321), Investments-Equity Method and Joint Ventures (Topic 323), and Derivatives and Hedging (Topic 815)*. ASU 2020-01 states any equity security transitioning from the alternative method of accounting under Topic 321 to the equity method, or vice versa, due to an observable transaction

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will be remeasured immediately before the transition. In addition, the ASU clarifies the accounting for certain non-derivative forward contracts or purchased call options to acquire equity securities stating such instruments will be measured using the fair value principles of Topic 321 before settlement or exercise. The ASU is effective for fiscal years beginning after December 15, 2020, and will be applied on a prospective basis. Early adoption is permitted. The Company is in the process of evaluating the impact of this new guidance on its consolidated financial statements and related disclosures.

In December 2019, the FASB issued ASU 2019-12, *Income Taxes (Topic 740) - Simplifying the Accounting for Income Taxes*. ASU 2019-12 simplifies the accounting for income taxes by removing exceptions within the general principles of Topic 740 regarding the calculation of deferred tax liabilities, the incremental approach for intraperiod tax allocation, and calculating income taxes in an interim period. In addition, the ASU adds clarifications to the accounting for franchise tax (or similar tax), which is partially based on income, evaluating tax basis of goodwill recognized from a business combination, and reflecting the effect of any enacted changes in tax laws or rates in the annual effective tax rate computation in the interim period that includes the enactment date. The ASU is effective for fiscal years beginning after December 15, 2020, and will be applied either retrospectively or prospectively based upon the applicable amendments. Early adoption is permitted. The Company is in the process of evaluating the impact of this new guidance on its consolidated financial statements and related disclosures.

In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820) Disclosure Framework - Changes to the Disclosure Requirements for Fair Value Measurement*. ASU 2018-13 resulted in certain modifications to fair value measurement disclosures, primarily related to level 3 fair value measurements. This standard was effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019, and early adoption was permitted. The adoption of this ASU did not have a material impact on the consolidated financial statements and related disclosures.

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments – Credit Losses (Topic 326) Measurement of Credit Losses on Financial Instruments*, which replaces the incurred loss impairment methodology in current GAAP with a methodology that reflects expected credit losses and requires consideration of a broader range of reasonable and supportable information to inform credit loss estimates. Additionally, ASU 2016-13 requires a financial asset measured at amortized cost basis to be presented at the net amount expected to be collected through the use of an allowance of expected credit losses. In May 2019, the FASB issued ASU 2019-05, *Financial Instruments - Credit Losses (Topic 326) Targeted Transition Relief*, which amends ASU 2016-13 by providing entities with an option to irrevocably elect the fair value option to be applied on an instrument-by-instrument basis for eligible financial instruments that are within the scope of Topic 326. The fair value option election does not apply to held-to-maturity debt securities. In November 2019, the FASB issued ASU 2019-10, *Financial Instruments - Credit Losses (Topic 326)*, *Derivatives and Hedging (Topic 815), and Leases (Topic 842)*, which finalized effective date delays for private companies, not-for-profit organizations, and certain smaller reporting companies applying the credit losses, leases, and hedging standards. Also, in November 2019, the FASB issued ASU 2019-11, *Codification Improvements to Topic 326, Financial Instruments - Credit Losses*, which provides clarity about certain aspects of the amendments in ASU 2016-13. ASU 2016-13, as amended, is effective for fiscal years beginning after December 15, 2022, including interim periods within those fiscal years, and requires a modified retrospective approach. The Company is in the process of evaluating the impact of this new guidance on its consolidated financial statements and related disclosures.

**3. Fair Value Measurements**

The following tables present the Company’s fair value hierarchy for assets and liabilities measured at fair value on a recurring basis (in thousands):

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **September 30, 2020** | | | | | | | | | | | | | | |
|  | | | **(Level 1)** | | |  | | | **(Level 2)** | | |  | | | **(Level 3)** | | |
| **Assets** | | |  | | |  | | |  | | |  | | |  | | |
| Money market funds (included in cash and cash equivalents) | | | $ | 37,635 |  |  | | | $ | — |  |  | | | $ | — |  |
|  | | |  | | |  | | |  | | |  | | |  | | |
|  | | |  | | |  | | |  | | |  | | |  | | |

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **December 31, 2019** | | | | | | | | | | | | | | |
|  | | | **(Level 1)** | | |  | | | **(Level 2)** | | |  | | | **(Level 3)** | | |
| **Assets** | | |  | | |  | | |  | | |  | | |  | | |
| Money market funds (included in cash and cash equivalents) | | | $ | 59,382 |  |  | | | $ | — |  |  | | | $ | — |  |
|  | | |  | | |  | | |  | | |  | | |  | | |
|  | | |  | | |  | | |  | | |  | | |  | | |

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**4. Accrued Expenses**

Accrued expenses consist of the following (amounts in thousands):

|  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **September 30, 2020** | | |  | | | **December 31, 2019** | | |
| Compensation and related benefits | | | $ | 1,602 |  |  | | | $ | 2,042 |  |
| Professional fees | | | 1,198 | |  |  | | | 2,929 | |  |
| Preclinical and clinical costs | | | 1,002 | |  |  | | | 1,820 | |  |
|  | | |  | | |  | | |  | | |
| Insurance premiums | | | 119 | |  |  | | | 1,423 | |  |
| Other | | | 346 | |  |  | | | 852 | |  |
| Total | | | $ | 4,267 |  |  | | | $ | 9,066 |  |

**5. Debt**

***Bpifrance Reimbursable Advance***

In December, 2017, in connection with its acquisition of Alizé Pharma SAS (“Alizé”), the Company assumed €0.7 million of debt that Alizé had outstanding with Bpifrance Financing (“Bpifrance”). The original advance amount of €0.8 million (“the Bpifrance Advance”) was provided to Alizé as an innovation aid that required Alizé to carry out certain activities related to its livoletide clinical development program and incur a certain level of program expenditures. No interest is charged or accrued under the advance.

The Company is required to make quarterly principal payments, which began in December 2016 and continue through September 2021. The quarterly principal payments escalate over the repayment period beginning with €17,500 per quarter and increasing to €50,000 through maturity. In addition to the quarterly payments, beginning January 1, 2016, Bpifrance may require the Company to pay, by no later than March 31 of each year, a reimbursement annuity equal to 20% of the proceeds generated by the Company from license, assignment or use of livoletide. Under no circumstance, however, would the Company be required to reimburse to Bpifrance principal amounts greater than the original advance it received.

The Company is permitted to repay the Bpifrance Advance at any time, at which point it would be released from all commitments and obligations under the Bpifrance Advance agreement. The Bpifrance Advance agreement does not contain any ongoing financial covenants.

During the three and nine months ended September 30, 2020 the Company made $53,000 in principal payments under the Bpifrance Advance agreement due to the fact that in April 2020, Bpifrance provided a six month deferral of principal payments to support businesses as a result of the COVID-19 pandemic. During the third quarter the Company resumed normal principal payments under the Bpifrance Advance agreement. During the three and nine months ended September 30, 2019, the Company made principal payments of $44,000 and $134,000, respectively. At September 30, 2020, the balance outstanding was $0.3 million (or €0.3 million).

**6. Commitments and Contingencies**

***Operating Leases***

The Company has noncancellable operating leases for office and laboratory space which have remaining lease terms between approximately two months and four years. In connection with privately-held Millendo Therapeutics, Inc.'s merger with OvaScience, Inc. in December 2018 (the “Merger”), the Company assumed a sublease agreement for office and laboratory space located in Waltham, Massachusetts. The sublease commenced on January 15, 2019 and expires in November 2020. The total minimum sublease rentals to be received under the Waltham, Massachusetts agreement is $0.6 million. The remaining sublease rentals to be received as of September 30, 2020 is $58,000. In February 2019 and October 2018, the Company entered into two additional noncancellable operating leases for office space in Ann Arbor, Michigan for the Company’s headquarters; one that the Company took possession of in April 2019, and the other that the Company took possession of in July 2019, respectively. One of its leases in Ann Arbor, Michigan expires in June 2024 and the other expires in March 2024. In April 2019, the Company entered into a lease agreement for office space in Lexington, Massachusetts. This lease was scheduled to expire on September 30, 2020; however, in June 2020 the Company exercised its right to terminate the lease early such that the lease terminated on August 11, 2020. Lease agreements generally do not require material variable lease payments, residual value guarantees or restrictive covenants. In January 2020, the Company terminated its office lease agreement in Lyon, France.

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As of September 30, 2020, the operating lease ROU asset and the operating lease liabilities were $2.4 million and $2.7 million, respectively. The weighted average discount rate used to account for the Company's operating leases under ASC 842 is the Company’s estimated incremental borrowing rate of 7.0%. The Company has options to extend certain of its leases for another five to ten years. These options to extend were not recognized as part of the Company’s measurement of the ROU assets and operating lease liabilities for the three and nine months ended September 30, 2020. The weighted average remaining term of the Company’s noncancellable operating leases is 3.43 years.

Rent expense related to the Company's operating leases was approximately $0.2 million and $0.2 million for the three months ended September 30, 2020 and 2019, respectively and approximately $0.7 million and $0.4 million for the nine months ended September 30, 2020 and 2019, respectively. The Company recognizes rent expense on a straight-lined basis over the lease period and has accrued for rent expense incurred but not yet paid.

Cash paid for amounts included in the measurement of the lease liabilities was approximately $0.5 million and $1.4 million during the three and nine months ended September 30, 2020, respectively. Cash paid for amounts included in the measurement of the lease liabilities was approximately $0.5 million and $1.0 million during the three and nine months ended September 30, 2019, respectively. The Company received approximately $87,000 and $0.3 million in sublease payments related to its Waltham, Massachusetts lease during the three and nine months ended September 30, 2020, respectively. The Company received approximately $86,000 and $0.2 million in sublease payments related to its Waltham, Massachusetts lease during the three and nine months ended September 30, 2019, respectively.

Future minimum rental payments under the Company’s noncancellable operating leases at September 30, 2020 is as follows (amounts in thousands):

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |
| 2020 (excluding the nine months ended September 30, 2020) | | | $ | 354 |  |
| 2021 | | | 760 | |  |
| 2022 | | | 783 | |  |
| 2023 | | | 806 | |  |
| 2024 | | | 302 | |  |
| Thereafter | | | — | |  |
| **Total** | | | $ | 3,005 |  |
| Present Value Adjustment | | | (320) | |  |
| **Lease liability at September 30, 2020** | | | $ | 2,685 |  |

***Litigation***

Liabilities for loss contingencies arising from claims, assessments, litigation, fines, penalties, and other sources are recorded when it is probable that a liability has been incurred and the amount can be reasonably estimated.

On November 9, 2016, a purported shareholder derivative action was filed in the Business Litigation Session of the Suffolk County Superior Court in the Commonwealth of Massachusetts (Cima v. Dipp, No. 16-3443-BLS1 (Mass. Sup. Ct.)) against certain former officers and directors of OvaScience and one current director of the Company (a former director of OvaScience) and OvaScience as a nominal defendant alleging breach of fiduciary duties, unjust enrichment, abuse of control, gross mismanagement and waste of corporate assets for purported actions related to OvaScience’s January 2015 follow-on public offering. On February 22, 2017, the court approved the parties’ joint stipulation to stay all proceedings in the action until further notice. Following a status conference in December 2017, the stay was lifted. On January 25, 2018, at the parties’ request, the court entered a second order staying all proceedings in the action until further order of the court. On March 2, 2020, the parties submitted a status report requesting that the court continue the stay. On March 5, 2020, the court entered an order continuing the stay and requiring that the parties file a further status report on or before June 30, 2020. On June 30, 2020, the parties filed a further status report requesting that the court continue the stay. The court has scheduled a conference for January 7, 2021 to review the status of the case. The Company believes that the complaint is without merit and intends to defend against the litigation. There can be no assurance, however, that the Company will be successful. At present, the Company is unable to estimate potential losses, if any, related to the lawsuit.

On March 24, 2017, a purported shareholder class action lawsuit was filed in the U.S. District Court for the District of Massachusetts (Dahhan v. OvaScience, Inc., No. 1:17-cv-10511-IT (D. Mass.)) against OvaScience and certain former officers

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of OvaScience alleging violations of Sections 10(b) and 20(a) of the Exchange Act (the “Dahhan Action”). On July 5, 2017, the court entered an order approving the appointment of Freedman Family Investments LLC as lead plaintiff, the firm of Robins Geller Rudman & Dowd LLP as lead counsel and the Law Office of Alan L. Kovacs as local counsel. Plaintiff filed an amended complaint on August 25, 2017. The Company filed a motion to dismiss the amended complaint, which the court denied on July 31, 2018. On August 14, 2018, the Company answered the amended complaint. On December 9, 2019, the court granted leave for the lead plaintiff to file a second amended complaint under seal and permitted the defendants to file a motion to strike the second amended complaint. On December 30, 2019, the court granted the parties’ joint motion to stay all proceedings in the case pending mediation. On March 3, 2020, the parties conducted a mediation session. The mediation was unsuccessful. The Company filed a motion to strike the second amended complaint on May 1, 2020. The Company believes that the amended complaint and the second amended complaint are without merit. The parties have agreed to participate in a second mediation session on November 10, 2020. A resolution of this lawsuit adverse to the Company or the other defendants could have a material effect on the Company's consolidated financial position and results of operations. At present, the Company is unable to estimate potential losses, if any, related to the lawsuit.

On July 27, 2017, a purported shareholder derivative complaint was filed in the U.S. District Court for the District of Massachusetts (Chiu v. Dipp, No. 1:17-cv-11382-IT (D. Mass.)) against OvaScience as a nominal defendant, certain former officers and directors of OvaScience and one current director of the Company (a former director of OvaScience) alleging breach of fiduciary duties, unjust enrichment and violations of Section 14(a) of the Exchange Act alleging that compensation awarded to the director defendants was excessive and seeking redress for purported actions related to OvaScience’s January 2015 follow-on public offering and other public statements concerning OvaScience's AUGMENT treatment. On September 26, 2017, the plaintiff filed an amended complaint which eliminated all claims regarding allegedly excessive director pay and additionally alleged claims of abuse of control and waste of corporate assets. On October 27, 2017, the defendants filed a motion to dismiss the amended complaint. The court heard oral argument on the motion to dismiss on April 5, 2018. On April 13, 2018, the court granted the defendants’ motion to dismiss the amended complaint for failure to state a claim for relief under Section 14(a). The court also dismissed the plaintiffs’ pendent state law claims without prejudice, based on lack of subject matter jurisdiction. On April 25, 2018, the plaintiffs moved for leave to amend the complaint and to stay this case pending the outcome of the Dahhan Action. The Company does not believe that the proposed amended complaint cures the defects in the current complaint, but informed plaintiffs’ counsel that, in the interest of judicial economy, defendants would not oppose the proposed amendment if the court would consider staying the case pending the resolution of the Dahhan Action. On April 27, 2018, the court granted the plaintiffs’ motion for leave to amend the complaint and for a stay. On April 30, 2018, the plaintiffs filed their second amended complaint. On May 23, 2018, the court entered an order staying this case pending the resolution of the Dahhan Action. the Company believes that the complaint is without merit and intend to defend against the litigation. There can be no assurance, however, that the Company will be successful. At present, the Company is unable to estimate potential losses, if any, related to the lawsuit.

In addition to the matters described above, the Company may be a party to litigation and subject to claims incident to the ordinary course of business from time to time. Regardless of the outcome, litigation can have an adverse impact on the Company because of defense and settlement costs, and diversion of management resources.

**7. Stock-Based Compensation**

On June 11, 2019, the Company held its 2019 Annual Meeting of Stockholders (the “Annual Meeting”). At the Annual Meeting, the Company’s stockholders approved the Company’s 2019 Equity Incentive Plan (the “2019 Plan”) and the Company’s 2019 Employee Stock Purchase Plan (the “2019 ESPP,” and together with the 2019 Plan, the “Plans”). The 2019 Plan is the successor to the Private Millendo 2012 Stock Plan and the OvaScience 2012 Stock Incentive Plan (each, as amended, the “Prior Plans”) and allows the Company to grant stock options, restricted stock unit awards and other awards at levels determined appropriate by the Company’s Board of Directors (the “Board”) or the Compensation Committee of the Board. No additional awards will be granted under either of the Prior Plans. The 2019 ESPP enables employees to purchase shares of the Company’s common stock through offerings of rights to purchase the Company’s common stock to all eligible employees. The Plans were adopted by the Board on April 29, 2019, subject to approval by the Company’s stockholders, and became effective with such stockholder approval on June 11, 2019. Outstanding awards under the Prior Plans continue to be subject to the terms and conditions of the Prior Plans.

The aggregate number of shares of the Company’s common stock initially reserved for issuance under the 2019 Plan was 2,919,872 shares, which is the sum of (i) 534,320 shares, (ii) the number of unallocated shares remaining available for grant under the Prior Plans as of the effective date of the 2019 Plan, and (iii) the Prior Plans’ Returning Shares (as defined below), as such shares become available from time to time. The number of shares of the Company's common stock reserved for issuance under the 2019 Plan will automatically increase on January 1 of each year, for a period of ten years, from January 1, 2020

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continuing through January 1, 2029, by 4% of the total number of shares of the Company's common stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares as may be determined by the Board.

The term “Prior Plan’s Returning Shares” refers to the following shares of the Company's common stock subject to any outstanding stock award granted under either of the Prior Plans: shares of common stock subject to awards that (i) expire or terminate for any reason prior to exercise or settlement; (ii) are forfeited because of the failure to meet a contingency or condition required to vest such shares or otherwise return to the Company; (iii) are reacquired, withheld (or not issued) to satisfy a tax withholding obligation in connection with an award or to satisfy the purchase price or exercise price of a stock award. The foregoing includes shares subject to outstanding awards under the OvaScience 2011 Stock Incentive 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 following shares of the Company’s common stock under the 2019 Plan (collectively, the “2019 Plan Returning Shares”) will also become available again for issuance under the 2019 Plan: (i) any shares subject to a stock award that are not issued because such stock award expires or otherwise terminates without all of the shares covered by such stock award having been issued, (ii) any shares subject to a stock award that are not issued because such stock award is settled in cash; (iii) any shares issued pursuant to a stock award that are forfeited back to or repurchased by the Company because of the failure to meet a contingency or condition required for the vesting of such shares; and (iv) any shares reacquired by the Company in satisfaction of tax withholding obligations on a stock award or as consideration for the exercise or purchase price of a stock award.

The aggregate number of shares of the Company’s common stock that may be issued under the 2019 ESPP is 133,580 shares, plus the number of shares of the Company’s common stock that are automatically added on January 1st of each year, for a period of up to ten years, from January 1, 2020 continuing through January 1, 2029, by the lesser of (i) 1% of the total number of shares of the Company's capital stock outstanding on December 31 of the preceding calendar year, or (ii) 133,580 shares of the Company's common stock, unless a lesser number of shares is determined by the Board. Pursuant to the terms of the 2019 Employee Stock Purchase Plan, an additional 133,580 shares were added to the number of available shares effective January 1, 2020.

The Company measures employee and nonemployee stock-based awards at grant date fair value and records compensation expense on a straight-line basis over the vesting period of the award.

The Company recorded stock-based compensation expense in the following expense categories of its accompanying consolidated statements of operations and comprehensive loss for the three months ended September 30, 2020 and 2019 and nine months ended September 30, 2020 and 2019, respectively (amounts in thousands):

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Three Months Ended September 30,** | | | | | | | | |  | | | **Nine Months Ended September 30,** | | | | | | | | |
|  | | | **2020** | | |  | | | **2019** | | |  | | | **2020** | | |  | | | **2019** | | |
| Research and development | | | $ | 258 |  |  | | | $ | 180 |  |  | | | $ | 731 |  |  | | | $ | 981 |  |
| General and administrative | | | 830 | |  |  | | | 1,016 | |  |  | | | 2,417 | |  |  | | | 2,149 | |  |
| Total | | | $ | 1,088 |  |  | | | $ | 1,196 |  |  | | | $ | 3,148 |  |  | | | $ | 3,130 |  |

***Stock options***

Options issued may have a contractual life of up to 10 years and may be exercisable in cash or as otherwise determined by the Board. Vesting generally occurs over a period of not greater than four years. In May 2020, the Company granted 840,450 stock options to its employees in connection with the PWS and CAH program changes that occurred during the second quarter of 2020 (see Note 1). The vesting is as follows: 1) 50 percent of the shares subject to this option grant will vest on the earlier of (i) December 31, 2020 or (ii) the Board's approval of the achievement of certain performance criteria; and 2) one twelfth (1/12th) of the remaining shares subject to this option grant will vest in equal monthly installments thereafter.

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The following table summarizes the activity related to stock option grants to employees and nonemployees for the nine months ended September 30, 2020:

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Shares** | | |  | | | **Weighted average exercise price per share** | | |  | | | **Weighted-average remaining contractual life (years)** | | |
| Outstanding at December 31, 2019 | | | 2,498,606 | |  |  | | | $ | 17.18 |  |  | | | 7.7 | | |
| Granted | | | 1,824,375 | |  |  | | | 4.77 | |  |  | | |  | | |
| Exercised | | | (1,449) | |  |  | | | 1.08 | |  |  | | |  | | |
| Forfeited | | | (567,356) | |  |  | | | 13.81 | |  |  | | |  | | |
| Outstanding at September 30, 2020 | | | 3,754,176 | |  |  | | | $ | 11.67 |  |  | | | 8.0 | | |
| Vested and exercisable at September 30, 2020 | | | 1,358,519 | |  |  | | | $ | 20.83 |  |  | | | 6.0 | | |
| Vested and expected to vest at September 30, 2020 | | | 3,754,176 | |  |  | | | $ | 11.67 |  |  | | | 8.0 | | |

As of September 30, 2020, the unrecognized compensation cost related to 2,395,657 unvested stock options expected to vest was $9.1 million. This unrecognized compensation will be recognized over an estimated weighted-average amortization period of 2.5 years. There were no stock options exercised during the three months ended September 30, 2020. The aggregate intrinsic value of options exercised during the nine months ended September 30, 2020 was $1,000. The aggregate intrinsic value of options exercised during the three and nine months ended September 30, 2019 was $0.5 million. The aggregate intrinsic value of both options outstanding and options exercisable as of September 30, 2020 was $44,000. The options granted during the three and nine months ended September 30, 2020 had an estimated weighted-average grant date fair value of $1.12 and $3.18, respectively. The grant date fair value of each option grant was estimated during the three and nine months ended September 30, 2020 and 2019 using the following assumptions within the Black-Scholes option-pricing model:

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Three Months Ended September 30, 2020** | | |  | | | **Three Months Ended September 30, 2019** | | |  | | | **Nine Months Ended September 30, 2020** | | |  | | | **Nine Months Ended September 30, 2019** | | |
| Expected term (in years) | | | 6.08 | | |  | | | 6.08 | | |  | | | 5.75 | | |  | | | 6.02 | | |
| Expected volatility | | | 77% | | |  | | | 81% | | |  | | | 77% | | |  | | | 80% | | |
| Risk-free interest rate | | | 0.36% | | |  | | | 1.51% | | |  | | | 0.87% | | |  | | | 2.23% | | |
| Expected dividend yield | | | 0% | | |  | | | 0% | | |  | | | 0% | | |  | | | 0% | | |

At the time of the Alizé acquisition, Alizé had 6,219 nonemployee (BSA) warrants and 5,360 employee (BSPCE) warrants outstanding, which have weighted-average exercise prices of €80.06 and €83.40, respectively. As of September 30, 2020, all BSA and BSPCE warrants were vested. During the three months ended September 30, 2020, no shares were exercised. During the nine months ended September 30, 2020, 910 BSPCE warrants were exercised resulting in the issuance of 12,307 shares of the Company’s common stock. In addition, during the three months ended September 30, 2020, there were no BSA and BSPCE warrants forfeited and during the nine months ended September 30, 2020, a total of 2,586 BSA and BSPCE warrants were forfeited. As of September 30, 2020, there were an aggregate of 48,265 shares of common stock issuable upon the exercise of the BSA and BSPCE warrants with a weighted-average exercise price of $7.49 per share. These instruments are included in the equity attributable to noncontrolling interests.

**8. Subsequent Events**

Subsequent events were evaluated through the filing date of this Quarterly Report on Form 10-Q.

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

*You should read the following discussion of our financial condition and results of operations in conjunction with our unaudited Interim Consolidated Financial Statements and the notes thereto included elsewhere in this Quarterly Report on Form 10-Q and with our annual audited Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2019 as filed with the Securities and Exchange Commission (“SEC”) on March 11, 2020. In addition to historical financial information, the following discussion contains forward-looking statements that reflect our plans, estimates, beliefs and expectations that involve risks and uncertainties. Our actual results and the timing of events could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to these differences include those discussed below and elsewhere in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K, particularly in Item 1A. “Risk Factors” and “Special Note Regarding Forward-Looking Statements.”*

**Overview**

We are a clinical stage biopharmaceutical company primarily focused on developing novel treatments for endocrine diseases where current therapies do not exist or are insufficient. We seek to leverage our understanding of recent biological discoveries in endocrinology to continue to advance and build our pipeline in order to improve the lives of patients. We are currently developing MLE-301, a selective neurokinin 3 receptor (NK3R) antagonist, as a potential treatment of vasomotor symptoms (“VMS”) in menopausal women. We are also actively pursuing additional pipeline assets in treatment areas where we have knowledge and experience in developing drug product candidates. We seek to identify assets that complement our current portfolio.

VMS are commonly known as hot flashes and night sweats in menopausal women. The sensations of heat and/or perspiration associated with VMS can occur frequently, generally lasting several minutes, and are often preceded or followed by sensations of cold and/or shivering. VMS interfere with the lives of affected women in a number of ways, including disrupting patients’ ability to sleep and concentrate and causing anxiety and depression. VMS are experienced by up to 70% of women as they advance through menopause. We believe that over 20 million women in the United States experience VMS at any given time and that these patients are motivated to seek medical treatment for relief.

In September 2020, we initiated our Phase 1 clinical trial of MLE-301. The first-in-human trial is designed to evaluate the safety and tolerability of MLE-301. The Phase 1 single ascending dose portion of the trial is being conducted in healthy male volunteers, to determine the pharmacokinetics of MLE-301 and its pharmacodynamic profile as measured by reductions of biomarkers (luteinizing hormone, testosterone). The Phase 1 multiple ascending dose portion will enroll post-menopausal women, with the goals of measuring reductions in VMS frequency and severity and establishing of initial clinical proof of concept. The Phase 1 clinical trial is supported by preclinical studies in which we observed potency and selectivity for the NK3R receptor, the potential for once-daily dosing, and testosterone lowering results consistent with the expected activity of an NK3R antagonist. We expect to initiate a Phase 2 clinical trial of MLE-301 in patients with VMS in 2021.

We had been developing livoletide (AZP-531) as a potential treatment for Prader-Willi syndrome (“PWS”), a rare and complex genetic endocrine disease characterized by hyperphagia, or insatiable hunger. As previously announced, we discontinued the development of livoletide as a potential treatment for PWS in April 2020, including the 9-month extension study and the initiation of the Phase 3 ZEPHYR trial. The decision to discontinue the PWS program was based on results from the Phase 2b ZEPHYR study, which showed that treatment with livoletide did not result in a statistically significant improvement in hyperphagia and food-related behaviors as measured by the Hyperphagia Questionnaire for Clinical Trials (HQ-CT) compared to placebo. We do not expect to incur future material expenses related to our livoletide program for the treatment of PWS.

In an effort to streamline costs after discontinuing our PWS program, we eliminated employee positions representing approximately 30% of our prior headcount, which were completed in the second quarter of 2020.

We had also been developing nevanimibe (ATR-101) as a potential treatment for patients with classic congenital adrenal hyperplasia (“CAH”), a rare, monogenic adrenal disease that requires lifelong treatment with exogenous cortisol, often at high doses. As we previously announced, we elected to cease investing in the development of nevanimibe as a potential treatment for CAH in June 2020. The decision to cease investment in the CAH program was based on the interim review of results from the Phase 2b clinical study and the changing competitive environment. Results from 10 subjects, nine from cohort 1 and one from cohort 2, with at least 12 weeks of treatment with nevanimibe in this open-label, continuous dose escalation study showed that one patient (10%) met the primary endpoint of achieving 17-hydroxyprogesterone (17-OHP) levels less than or equal to 2-times the upper limit of normal. Treatment under the amended protocol with dose titration starting at 500 mg BID improved tolerability of nevanimibe. We are currently exploring the option of out-licensing nevanimibe. We do not expect to incur future material expenses related to our nevanimibe program for the treatment of CAH.

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In connection with the discontinuation of our livoletide program in PWS and the end of our investment in our nevanimibe program in CAH, we continue to evaluate our business strategy to prioritize and allocate resources towards the advancement of our current product candidate, MLE-301, and any potential future pipeline assets. As part of these efforts, we have engaged SVB Leerink to support our strategic review process, which is intended to result in an actionable plan that leverages our assets, capital and capabilities to maximize stockholder value.

Since inception, we have incurred significant operating losses and negative operating cash flows and there is no assurance that we will ever achieve or sustain profitability. Our net losses were $6.4 million and $11.6 million for the three months ended September 30, 2020 and 2019, respectively, and $29.0 million and $31.9 million for the nine months ended September 30, 2020 and 2019, respectively. As of September 30, 2020, we had an accumulated deficit of $237.7 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future.

**COVID-19 Business Update**

With the global impacts of the ongoing COVID-19 pandemic continuing in the third quarter of 2020, we are maintaining the cross-functional task force and business continuity plans we established and implemented in the first quarter of 2020, which are designed to address and mitigate the impact of the COVID-19 pandemic on our employees, operations and our business. While we are experiencing limited financial impacts from the pandemic at this time, given the global economic slowdown, the overall disruption of global healthcare systems and the other risks and uncertainties associated with the pandemic, our business, financial condition, results of operations, growth prospects, preclinical studies and clinical development programs and timelines, including our ongoing Phase 1 clinical trial of MLE-301, could be materially adversely affected. We continue to closely monitor the COVID-19 situation as we evolve our business continuity plans and response strategy. In March 2020, our global workforce transitioned to working remotely. Throughout the third quarter of 2020, we continued our plan to allow some employees to return to the office voluntarily, which was based on a phased approach that is principles-based, flexible and local in design, with a focus on employee safety and optimal work environment. Our current plans remain fluid as federal, state and local guidelines, rules and regulations continue to evolve.

***Supply Chain***

We are working closely with our third-party manufacturers, distributors and other partners to manage our supply chain activities and mitigate potential disruptions to our product supplies as a result of the COVID-19 pandemic. We currently expect to have adequate global supply of MLE-301 to support our ongoing preclinical studies and our ongoing Phase 1 clinical trial, which we initiated in the third quarter of 2020. If the COVID-19 pandemic persists for an extended period of time and further impacts essential distribution systems such as express package and postal delivery, we could experience disruptions to our supply chain and operations, and associated delays in the manufacturing and supply of our products, which would adversely impact our ability to conduct clinical and preclinical trials.

***Clinical Development***

With respect to clinical development, we are prepared to take measures as needed to implement remote and virtual approaches, including remote patient monitoring and home delivery of drug treatments where possible, to maintain patient safety and trial continuity and to preserve study integrity. As the COVID-19 pandemic continues, it is possible that there will be an impact on our ability to initiate trial sites, enroll and assess patients and maintain patient enrollment, including in the ongoing Phase 1 clinical trial of MLE-301. We could also see an impact on our ability to acquire supplies of study drug, report trial results or interact with regulators, ethics committees or other important agencies due to limitations in regulatory authority employee resources or otherwise. In addition, we rely on contract research organizations or other third-parties to assist us with clinical trials, and we cannot guarantee that they will continue to perform their contractual duties in a timely and satisfactory manner as a result of the COVID-19 pandemic. If the COVID-19 pandemic continues and persists for an extended period of time, we could experience significant disruptions to our clinical development timelines, which would adversely affect our business, financial condition, results of operations and growth prospects.

***Regulatory Activities***

We have not experienced, to date, any significant delays with respect to regulatory reviews or interactions with regulatory authorities as a result of the COVID-19 pandemic. Neither the U.S. Food and Drug Administration (the "FDA") or other regulatory authorities such as the European Medicines Agency (the "EMA"), has notified us of any COVID-19-related delays in reviews impacting our clinical or preclinical programs. However, it is possible that we could experience substantial delays in the timing of regulatory reviews or interactions with the FDA, EMA, or other regulatory authorities due to, for example,

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absenteeism by governmental employees or the diversion of regulators' efforts and attention to approval of other therapeutics or other activities related to COVID-19.

***Corporate Development***

With our strong cash balance, we anticipate having sufficient liquidity to make strategic investments in our business this year in support of our long-term growth strategy. We believe that our cash, cash equivalents and restricted cash as of September 30, 2020 will fund our planned operations into 2022. However, our operating plan has recently changed due to discontinuations and revaluations of our clinical trial programs and may change further as a result of our ongoing strategic review or other factors currently unknown to us. We may need to seek additional funds sooner than planned, through public or private equity or debt financings, third-party funding, marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or any combination of these approaches. In addition, the COVID-19 pandemic continues to evolve and has already resulted in a significant disruption of global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital, which could in the future negatively affect our operations.

***Other Financial and Corporate Impacts***

Although we have experienced limited financial impacts from the pandemic at this time, we expect that the COVID-19 pandemic could adversely affect our business operations and financial results, our clinical development and regulatory efforts, our corporate development objectives and the value of and market for our common stock, which will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the U.S., Europe and other geographies, and the effectiveness of actions taken globally to contain and treat the disease. For example, if remote work policies for certain portions of our business, or that of our business partners, are extended longer than we currently expect, we may need to reassess our priorities and our corporate objectives for the year.

We have not experienced any material impact to our internal controls over financial reporting despite the fact that some of our employees are working remotely due to the COVID-19 pandemic. We are continually monitoring and assessing the COVID-19 situation on our internal controls to minimize the impact on their design and operating effectiveness. In addition, all information technology operations are inherently vulnerable to inadvertent or intentional security breaches, incidents, attacks and exposures, the accessibility and distributed nature of our information technology systems, and the sensitive information stored on those systems, make such systems potentially vulnerable to unintentional or malicious, internal and external attacks on our technology environment. Due to the COVID-19 pandemic, we have enabled all of our employees to work remotely, which may make us more vulnerable to cyberattacks or other incidents. To date, we have not experienced any increase in cyberattacks or other incidents.

**Components of Our Results of Operations**

***Research and development expense***

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

•personnel expenses, including salaries, benefits and stock-based compensation expense;

•costs of funding research performed by third-parties, including pursuant to agreements with contract research organizations, (“CROs”), as well as investigative sites and consultants that conduct our preclinical studies and clinical trials;

•expenses incurred under agreements with contract manufacturing organizations (“CMOs”), including manufacturing scale-up expenses and the cost of acquiring and manufacturing preclinical study and clinical trial materials;

•payments made under our third-party licensing agreements;

•consultant fees and expenses associated with outsourced professional scientific development services;

•expenses for regulatory activities, including filing fees paid to regulatory agencies; and

•allocated expenses for facility costs, including rent, utilities, depreciation and maintenance.

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Milestone payment obligations incurred prior to regulatory approval of a product candidate, which are accrued when the event requiring payment of the milestone occurs are included in research and development expense.

We typically use our employee, consultant and infrastructure resources across our development programs. We track certain outsourced development costs by product candidate, but do not allocate all personnel costs or other internal costs to specific product candidates.

The following table summarizes our research and development expenses by product candidate, personnel expense and other expenses for the three and nine months ended September 30, 2020 and 2019, respectively:

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Three Months Ended September 30,** | | | | | | | | |  | | | **Nine Months Ended September 30,** | | | | | | | | |
|  | | | **2020** | | |  | | | **2019** | | |  | | | **2020** | | |  | | | **2019** | | |
|  | | | **(dollars in thousands)** | | | | | | | | |  | | | **(dollars in thousands)** | | | | | | | | |
| Livoletide expenses | | | $ | 83 |  |  | | | $ | 3,850 |  |  | | | $ | 8,053 |  |  | | | $ | 9,881 |  |
| Nevanimibe expenses | | | 145 | |  |  | | | 867 | |  |  | | | 882 | |  |  | | | 2,595 | |  |
| MLE-301 expenses | | | 1,312 | |  |  | | | 838 | |  |  | | | 2,739 | |  |  | | | 1,282 | |  |
| Personnel expenses | | | 1,006 | |  |  | | | 1,529 | |  |  | | | 4,515 | |  |  | | | 5,054 | |  |
| Other expenses | | | 130 | |  |  | | | 224 | |  |  | | | 493 | |  |  | | | 681 | |  |
| Total | | | $ | 2,676 |  |  | | | $ | 7,308 |  |  | | | $ | 16,682 |  |  | | | $ | 19,493 |  |

Our research and development costs related to livoletide and nevanimibe have decreased significantly due to our decision to discontinue the livoletide program and our decision to cease investing in the development of nevanimibe based on results from the Phase 2b ZEPHYR study in PWS and the Phase 2b clinical study in CAH, respectively. All costs, including estimated program closeout costs associated with these programs, were recognized during the second quarter of 2020. Any revisions to estimated program closeout costs have been recognized as of September 30, 2020. Future expenses may be recorded as a result of changes to these estimated costs as closeout activities continue. We expect our research and develop costs related to MLE-301 to increase as we continue preclinical studies and clinical trials, including our ongoing Phase 1 clinical trial of MLE-301.

The successful development of our current product candidate or any future 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 remainder of the development of MLE-301. We are also unable to predict when, if ever, material net cash inflows may commence from sales of MLE-301 or any future product candidates that we may develop due to the numerous risks and uncertainties associated with clinical development, including risks and uncertainties related to:

•the ongoing COVID-19 pandemic, including the potential impact on various aspects and stages of the clinical development process;

•the number of clinical sites included in the trials;

•the length of time required to enroll suitable patients;

•the number of patients that ultimately participate in the trials;

•the number of doses patients receive;

•the duration of patient follow-up and number of patient visits;

•the results of our clinical trials;

•the establishment of commercial manufacturing capabilities;

•the receipt of marketing approvals; and

•the commercialization of product candidates.

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We may never succeed in obtaining regulatory approval for MLE-301 or any future product candidates we may develop. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials.

***General and administrative expense***

General and administrative expense consists primarily of personnel expenses, including salaries, benefits and stock-based compensation expense, for employees in executive, finance, accounting, business development, legal and human resource functions. General and administrative expense also includes corporate facility costs, including rent, utilities, depreciation and maintenance, not otherwise included in research and development expense, as well as legal fees related to intellectual property and corporate matters and fees for accounting, recruiting and consulting services.

***Interest expense (income), net***

Interest expense (income) represents amounts earned on our cash, cash equivalents and restricted cash balances.

***Results of operations***

***Comparison of the three months ended September 30, 2020 and 2019***

The following table summarizes our operating results for the periods indicated:

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Three Months Ended September 30,** | | | | | | | | |  | | |  | | |  | | |  | | |
|  | | | **2020** | | |  | | | **2019** | | |  | | | **Change** | | | | | | | | |
|  | | | **(dollars in thousands)** | | | | | | | | | | | | | | | | | | | | |
| Operating expenses: | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Research and development | | | $ | 2,676 |  |  | | | $ | 7,308 |  |  | | | $ | (4,632) |  |  | | | (63.4) | | % |
| General and administrative | | | 3,380 | |  |  | | | 4,443 | |  |  | | | (1,063) | |  |  | | | (23.9) | |  |
| Loss from operations | | | 6,056 | |  |  | | | 11,751 | |  |  | | | (5,695) | |  |  | | | (48.5) | |  |
| Other expenses (income): | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Interest expense (income), net | | | 8 | |  |  | | | (238) | |  |  | | | 246 | |  |  | | | (103.4) | |  |
| Other loss | | | 310 | |  |  | | | 119 | |  |  | | | 191 | |  |  | | | 160.5 | |  |
| Net loss | | | $ | (6,374) |  |  | | | $ | (11,632) |  |  | | | $ | 5,258 |  |  | | | (45.2) | | % |

***Research and development expense***

Research and development expense decreased by $4.6 million to $2.7 million for the three months ended September 30, 2020 from $7.3 million for the three months ended September 30, 2019. The following table summarizes our research and development expenses for the three months ended September 30, 2020 and 2019:

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Three Months Ended September 30,** | | | | | | | | |  | | |  | | |  | | |  | | |
|  | | | **2020** | | |  | | | **2019** | | |  | | | **Change** | | | | | | | | |
|  | | | **(dollars in thousands)** | | | | | | | | | | | | | | | | | | | | |
| Preclinical and clinical development expense | | | $ | 1,540 |  |  | | | $ | 5,555 |  |  | | | $ | (4,015) |  |  | | | (72.3) | | % |
| Compensation expense, other than stock-based compensation | | | 748 | |  |  | | | 1,349 | |  |  | | | (601) | |  |  | | | (44.6) | |  |
| Stock-based compensation expense | | | 258 | |  |  | | | 180 | |  |  | | | 78 | |  |  | | | 43.3 | |  |
| Other expenses | | | 130 | |  |  | | | 224 | |  |  | | | (94) | |  |  | | | (42.0) | |  |
| Total research and development expense | | | $ | 2,676 |  |  | | | $ | 7,308 |  |  | | | $ | (4,632) |  |  | | | (63.4) | | % |

The decrease in total research and development expense is attributable to:

•a $4.0 million decrease in preclinical and clinical development expense primarily related to decreased spend due to discontinuing our development of the livoletide program and ceasing investment in the nevanimibe programs offset by increased spend on MLE-301;

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•a $0.6 million decrease in compensation expense, other than stock-based compensation primarily due to the reduction in force completed in the second quarter of 2020, as a result of the discontinuance of our livoletide program; and

•a $0.1 million increase in stock-based compensation expenses primarily related to additional options granted in 2020.

***General and administrative expense***

General and administrative expense decreased by $1.1 million to $3.4 million for the three months ended September 30, 2020 from $4.4 million for the three months ended September 30, 2019. The decrease was primarily due to lower professional fees and compensation expenses, including stock-based compensation. Professional fees decreased $0.8 million as a result of lower accounting fees and consulting fees incurred as compared to the prior period. The decrease in these fees was due to decreased expenditure on preparations for certain public reporting requirements in 2020 as compared to 2019, as well as lower consulting fees incurred related to assessing market opportunities for previous product candidates. Compensation and stock-based compensation decreased by $0.3 million as a result of a decrease in our general and administrative headcount and changes to compensation arrangements related to our reduction in force in the second quarter of 2020.

***Interest expense (income), net***

Interest expense (income), net decreased by $0.2 million to $8,000 interest expense, net for the three months ended September 30, 2020 from interest income, net of $0.2 million for the three months ended September 30, 2019. The change was primarily due to lower interest income received as a result of lower cash and cash equivalent and marketable securities balances and lower interest rates.

***Other loss***

Other loss increased by $0.2 million to $0.3 million for the three months ended September 30, 2020 from $0.1 million for the three months ended September 30, 2019 due to higher foreign currency losses as a result of exchange rate fluctuations on transactions denominated in a currency other than our functional currency.

***Comparison of the nine months ended September 30, 2020 and 2019***

The following table summarizes our operating results for the periods indicated:

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Nine Months Ended September 30,** | | | | | | | | |  | | |  | | |  | | |  | | |
|  | | | **2020** | | |  | | | **2019** | | |  | | | **Change** | | | | | | | | |
|  | | | **(dollars in thousands)** | | | | | | | | | | | | | | | | | | | | |
| Operating expenses: | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Research and development | | | $ | 16,682 |  |  | | | $ | 19,493 |  |  | | | $ | (2,811) |  |  | | | (14.4) | | % |
| General and administrative | | | 12,113 | |  |  | | | 13,075 | |  |  | | | (962) | |  |  | | | (7.4) | |  |
| Loss from operations | | | 28,795 | |  |  | | | 32,568 | |  |  | | | (3,773) | |  |  | | | (11.6) | |  |
| Other expenses (income): | | |  | | |  | | |  | | |  | | |  | | |  | | |  | | |
| Interest income, net | | | (159) | |  |  | | | (866) | |  |  | | | 707 | |  |  | | | (81.6) | |  |
| Other loss | | | 408 | |  |  | | | 167 | |  |  | | | 241 | |  |  | | | 144.3 | |  |
| Net loss | | | $ | (29,044) |  |  | | | $ | (31,869) |  |  | | | $ | 2,825 |  |  | | | (8.9) | | % |

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***Research and development expense***

Research and development expense decreased by $2.8 million to $16.7 million for the nine months ended September 30, 2020 from $19.5 million for the nine months ended September 30, 2019. The following table summarizes our research and development expenses for the nine months ended September 30, 2020 and 2019:

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Nine Months Ended September 30,** | | | | | | | | |  | | |  | | |  | | |  | | |
|  | | | **2020** | | |  | | | **2019** | | |  | | | **Change** | | | | | | | | |
|  | | | **(dollars in thousands)** | | | | | | | | | | | | | | | | | | | | |
| Preclinical and clinical development expense | | | $ | 11,674 |  |  | | | $ | 13,758 |  |  | | | $ | (2,084) |  |  | | | (15.1) | | % |
| Compensation expense, other than stock-based compensation | | | 3,784 | |  |  | | | 4,073 | |  |  | | | (289) | |  |  | | | (7.1) | |  |
| Stock-based compensation expense | | | 731 | |  |  | | | 981 | |  |  | | | (250) | |  |  | | | (25.5) | |  |
| Other expenses | | | 493 | |  |  | | | 681 | |  |  | | | (188) | |  |  | | | (27.6) | |  |
| Total research and development expense | | | $ | 16,682 |  |  | | | $ | 19,493 |  |  | | | $ | (2,811) |  |  | | | (14.4) | | % |

The decrease in total research and development expense is attributable to:

•a $2.1 million decrease in preclinical and clinical development expense primarily related to decreased spend due to discontinuing our development of the livoletide and nevanimibe programs offset by increased spend on MLE-301;

•a $0.3 million decrease in compensation expense, other than stock-based compensation primarily due to the reduction in force completed in the second quarter of 2020, as a result of the discontinuance of the livoletide program; and

•a $0.3 million decrease in stock-based compensation expenses primarily related to the reduction in force completed in the second quarter of 2020.

***General and administrative expense***

General and administrative expense decreased by $1.0 million to $12.1 million for the nine months ended September 30, 2020 from $13.1 million for the nine months ended September 30, 2019. The decrease was primarily due to $2.0 million decrease in professional fees primarily as a result of lower legal and accounting fees incurred as compared to the prior period. The decrease in professional fees was due to decreased expenditure on preparations for certain public reporting requirements in 2020 as compared to 2019, as well as lower consulting fees incurred related to assessing market opportunities for previous product candidates. These decreases were partially offset by a $0.8 million increase in compensation and stock-based compensation expense primarily as a result of termination benefits paid in connection with our reduction in force in the second quarter of 2020 and additional options granted in 2020. In addition there was an increase of $0.2 million in other related expenses.

***Interest income, net***

Interest income, net decreased by $0.7 million to $0.2 million interest income, net for the nine months ended September 30, 2020 from interest income, net of $0.9 million for the nine months ended September 30, 2019. The change was primarily due to lower interest income received as a result of lower cash and cash equivalent and marketable securities balances and lower interest rates.

***Other loss***

Other loss increased by $0.2 million to $0.4 million for the nine months ended September 30, 2020 from $0.2 million for the nine months ended September 30, 2019 due to higher foreign currency losses as a result of exchange rate fluctuations on transactions denominated in a currency other than our functional currency.

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**Liquidity and Capital Resources**

***Cash flows***

The following table sets forth the primary uses of cash and cash equivalents for the nine months ended September 30, 2020 and 2019:

|  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |  |  |  |
|  | | | **Nine Months Ended September 30,** | | | | | | | | |
|  | | | **2020** | | |  | | | **2019** | | |
|  | | | **(in thousands)** | | | | | | | | |
| Net cash used in operating activities | | | $ | (25,132) |  |  | | | $ | (29,531) |  |
| Net cash (used in) provided by investing activities | | | (26) | |  |  | | | 4,021 | |  |
| Net cash provided by financing activities | | | 5,450 | |  |  | | | 83 | |  |
| Effect of foreign currency exchange rate changes on cash | | | (52) | |  |  | | | 5 | |  |
| Net decrease in cash, cash equivalents and restricted cash | | | $ | (19,760) |  |  | | | $ | (25,422) |  |

*Operating activities*

During the nine months ended September 30, 2020, we used $25.1 million of cash to fund operating activities. During the nine months ended September 30, 2020, cash used in operating activities reflected our net loss of $29.0 million offset by non-cash charges of $4.4 million, principally related to stock-based compensation, amortization of our right-of-use assets and the foreign currency remeasurement loss.

During the nine months ended September 30, 2019, we used $29.5 million of cash to fund operating activities. During the nine months ended September 30, 2019, cash used in operating activities reflected our net loss of $31.9 million and a net change in operating assets and liabilities of $1.6 million, offset by non-cash charges of $3.9 million, principally related to stock-based compensation and amortization of our right-of-use assets.

*Investing activities*

During the nine months ended September 30, 2020, we paid $26,000 in purchases of property and equipment. During the nine months ended September 30, 2019, we received $4.4 million in net proceeds from the sale of marketable securities and paid $0.4 million in purchases of property and equipment.

*Financing activities*

During the nine months ended September 30, 2020, we received proceeds of $5.7 million received from the issuance of common stock, net of issuance costs paid. See Note 1 of our Unaudited Interim Consolidated Financial Statements for additional information related to the issuance of common stock. These proceeds were offset by $0.2 million in the payment of financing costs. During the nine months ended September 30, 2019, we received proceeds of $0.5 million from the exercise of options and warrants, which were offset by $0.1 million for the repayment of debt, and $0.2 million in the payment of financing costs.

***Funding requirements***

We expect our expenses to decrease as a result of our discontinuing the development of livoletide and our ceasing investment in the development of nevanimibe as compared to previous operations. We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development, the continuation of clinical trials and seek marketing approval for, our current or any future product candidates. Furthermore, we expect to continue to incur costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts. The COVID-19 pandemic continues to rapidly evolve and has already resulted in a significant disruption of global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital, which could in the future negatively affect our operations.

In April 2019, we entered into an “at-the-market” (“ATM”), equity distribution agreement with Citigroup Global Markets Inc. acting as sole agent with an aggregate offering value of up to $50.0 million. Subject to the terms of the ATM equity distribution

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agreement, we are able to determine, at our sole discretion, the timing and number of shares to be sold under this ATM facility. In March 2020, we amended and restated the equity distribution agreement to include SVB Leerink LLC as an additional sales agent for the ATM. In March 2020, we sold 719,400 shares of common stock under our ATM equity distribution agreement for net proceeds of approximately $5.7 million.

As of September 30, 2020, we had cash, cash equivalents and restricted cash of $43.8 million, which we believe are sufficient to fund our planned operations into 2022. This cash runway guidance is based on our current operational plans and excludes any additional funding that may be received and business development activities that may be undertaken. In addition, our operating plans may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, third-party funding, and marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or a combination of these approaches. In any event, we will require additional capital to pursue regulatory approval and the commercialization of our current and future product candidates.

Our future capital requirements will depend on many factors, including:

•the scope, progress, results and costs of preclinical studies and clinical trials;

•the scope, prioritization and number of our research and development programs;

•the costs, timing and outcome of regulatory review of our product candidates;

•our ability to establish and maintain collaborations on favorable terms, if at all;

•the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under collaboration agreements, if any;

•the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;

•the extent to which we acquire or in-license other product candidates and technologies;

•the costs of securing manufacturing arrangements for commercial production; and

•the costs of establishing or contracting for sales and marketing capabilities if we obtain regulatory approvals to market our product candidates.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes many 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 commercial revenues, if any, will be derived from sales of product candidates that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise funds through additional collaborations, strategic alliances 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 through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

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**Contractual Obligations and Commitments**

In January 2020, we terminated our office lease agreement in Lyon, France and in August 2020, we terminated our lease in Lexington, MA.

During the three and nine months ended September 30, 2020, there were no other material changes to our contractual obligations and commitments described under “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our Annual Report on Form 10-K.

**Off-Balance Sheet Arrangements**

We did not have any off-balance sheet arrangements as of September 30, 2020, as defined in Item 303(a)(4)(ii) of Regulation S-K.

**Critical Accounting Policies and Estimates**

Other than as described under Note 2 to our Unaudited Interim Consolidated Financial Statements, the Critical Accounting Policies included in our Annual Report on Form 10-K for the year ended December 31, 2019, as filed with the SEC on March 11, 2020, have not materially changed.

**Item 3. *Quantitative and Qualitative Disclosures about Market Risk***

Not required for smaller reporting companies.

**Item 4. *Controls and Procedures***

**Evaluation of Disclosure Controls and Procedures**

Our management, with the participation of our Chief Executive Officer (principal executive officer) and Chief Financial Officer (principal financial officer), evaluated the effectiveness of our disclosure controls and procedures (as defined in the Securities Exchange Act of 1934 Rules 13a-15(e) or 15d-15(e)) as required by paragraph (b) of Exchange Act Rules 13a-15 or 15d-15, as of September 30, 2020. Based on the evaluation of our disclosure controls and procedures as of September 30, 2020, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at a reasonable assurance level.

**Changes in Internal Control over Financial Reporting**

There were no changes in internal control over financial reporting during the quarter ended September 30, 2020 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting. We have not experienced any material impact to our internal controls over financial reporting despite the fact that our employees are working remotely due to the COVID-19 pandemic. We are continually monitoring and assessing the COVID-19 situation on our internal controls to minimize the impact on their design and operating effectiveness.

**Inherent Limitations on Effectiveness of Controls**

Our management, including our Chief Executive Officer and our Chief Financial Officer, believes that our disclosure controls and procedures and internal control over financial reporting are designed to provide reasonable assurance of achieving their objectives and are effective at the reasonable assurance level. However, our management does not expect that our disclosure controls and procedures or our internal control over financial reporting will prevent all errors and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, have been detected. These inherent limitations include the realities that judgments in decision making can be faulty, and that breakdowns can occur because of a simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by management override of the controls. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes

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in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

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**PART II**

**Item 1. *Legal Proceedings***

Other than as described under Note 6 to our Unaudited Interim Consolidated Financial Statements, the Legal Proceedings included in our Annual Report on Form 10-K for the year ended December 31, 2019, as filed with the SEC on March 11, 2020, have not materially changed.

**Item 1A. *Risk Factors***

You should carefully consider the risks described below, as well as general economic and business risks and the other information in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for the year ended December 31, 2019. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment. We cannot assure you that any of the events discussed below will not occur. Such risks may be amplified by the COVID-19 pandemic and its potential impact on our business and the global economy.

**Risks Related to Our Financial Position and Need for Additional Capital**

***We have incurred significant operating losses since inception and anticipate that we will continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.***

Since inception, we have incurred significant operating losses and negative operating cash flows and there is no assurance that we will ever achieve or sustain profitability. Our net loss was $27.2 million and $44.6 million for the years ended December 31, 2018 and 2019, respectively and $6.4 million and $29.0 million for the three and nine months ended September 30, 2020, respectively. As of September 30, 2020, we had an accumulated deficit of $237.7 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We have devoted substantially all of our efforts to the acquisition of and preclinical and clinical development of MLE-301, our current product candidate, nevanimibe, which we ceased our investment in the development of as a potential treatment of patients with CAH in June 2020, and livoletide, which we discontinued the development of as a potential treatment of patients with PWS in April 2020, as well as to building our management team and infrastructure. It could be several years, if ever, before we have a commercialized product and our commercialized products, if any, may not be profitable. The net losses we incur may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase significantly in connection with our ongoing activities such as:

***•***continue our ongoing and planned development of MLE-301 for the treatment of vasomotor symptoms (“VMS”) in menopausal women, including our Phase 1 clinical trial of MLE-301;

•initiating preclinical studies and clinical trials for any additional diseases for our current product candidates and any future product candidates that we may pursue;

•building a portfolio of product candidates through acquisition or in-licensing;

•developing, maintaining, expanding and protecting our intellectual property portfolio;

•manufacturing, or having manufactured, clinical and commercial supplies of our product candidates;

•seeking marketing approvals for our current and future product candidates that successfully complete clinical trials;

•establishing a sales, marketing and distribution infrastructure to commercialize any product candidate for which we may obtain marketing approval;

•hiring additional administrative, clinical, regulatory and scientific personnel; and

•continuing to incur additional costs associated with operating as a public company.

In order to become and remain profitable, we will need to develop and eventually commercialize, on our own or with collaborators, one or more product candidates with significant market potential. This will require us to be successful in a range

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of challenging activities, including completing clinical trials of MLE-301 and potential future product candidates, developing commercial scale manufacturing processes, obtaining marketing approval, manufacturing, marketing and selling any current and future product candidates for which we may obtain marketing approval, and satisfying any post-marketing requirements. We are only in the early stages of some of these activities, and may never succeed in any or all of these activities and, even if we do, we may never generate revenue from product sales or achieve profitability. For example, we discontinued our development of livoletide as a potential treatment for PWS in April 2020 based on topline results from the Phase 2b ZEPHYR trial which showed that treatment with livoletide did not result in a statistically significant improvement in hyperphagia and food-related behaviors as measured by the HQ-CT compared to placebo. In addition, we do not currently plan further investment in the development of nevanimibe for the treatment of CAH, based on the interim data review of the Phase 2b trial completed in June 2020.

Because of the numerous risks and uncertainties associated with pharmaceutical products and development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. If we are required by the U.S. Food and Drug Administration (the "FDA") or other regulatory authorities, such as the European Medicines Agency (the "EMA"), to amend existing studies or trials, to perform studies in addition to those currently expected, or if there are any delays in the development or in the completion of any planned or future preclinical studies or clinical trials of our current or future product candidates, our expenses could increase and profitability could be further delayed.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease our value and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in our value also could cause you to lose all or part of your investment.

***We have a limited operating history and have never generated any revenue from product sales, which may make it difficult to assess our future viability.***

We are a clinical stage biopharmaceutical company with a limited operating history. Our operations to date, with respect to the development of our product candidates, have been limited to organizing and staffing the business, business planning, raising capital, acquiring our product candidates and other assets and conducting preclinical and clinical development of our product candidates. We have not yet demonstrated an ability to successfully complete clinical development of a product candidate, obtain marketing approval, manufacture a commercial-scale drug (or arrange for a third-party to do so on our behalf), or conduct sales and marketing activities necessary for successful commercialization. Consequently, our predictions about our future success or viability may not be as accurate as they could be if we had more experience developing product candidates.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with any future collaborations, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize MLE-301 and any additional product candidates that we may pursue in the future. We do not anticipate generating revenue from product sales for the next several years, if ever. Our ability to generate revenue from product sales depends heavily on our or any future collaborators’ success in:

•timely and successful completion of development activities of our current product candidates;

•obtaining and maintaining regulatory and marketing approvals for MLE-301 and any future product candidates for which we successfully complete clinical trials;

•launching and commercializing any product candidates for which we obtain regulatory and marketing approval by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;

•qualifying for coverage and adequate reimbursement by government and third-party payors for our current or any future product candidates, if approved, both in the United States and internationally, and reaching acceptable agreements with such government and third-party payors on pricing terms;

**•**developing, validating and maintaining a commercially viable, sustainable, scalable, reproducible and transferable manufacturing process for MLE-301 or any future product candidates that are compliant with current good manufacturing practices (“cGMP”);

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•establishing and maintaining supply and manufacturing relationships with third-parties that can provide an adequate amount and quality of our product candidates and services to support our planned clinical development, as well as the market demand for MLE-301 and any future product candidates, if approved;

•obtaining market acceptance, if and when approved, of MLE-301 or any future product candidates as a viable treatment option by physicians, patients, third-party payors and others in the medical community;

•effectively addressing any competing technological and market developments;

•implementing additional internal systems and infrastructure, as needed;

•negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter, and performing our obligations pursuant to such arrangements;

•maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;

•avoiding and defending against third-party interference or infringement claims; and

•attracting, hiring and retaining qualified personnel.

***We will require additional capital to finance our operations, which may not be available on acceptable terms, if at all. Failure to obtain capital when needed may force us to delay, limit or terminate certain of our development programs, future commercialization efforts or other operations.***

We expect our expenses to increase in connection with our ongoing activities, particularly as we conduct our Phase 1 clinical trial of MLE-301 and continue to develop, and if approved, commercialize MLE-301. Additionally, if we obtain marketing approval for our product candidates, we expect to incur significant expenses related to manufacturing, marketing, sales and distribution. Furthermore, we expect to continue to incur additional costs associated with operating as a public company.

As of September 30, 2020, our cash, cash equivalents and restricted cash were $43.8 million. Our existing cash, cash equivalents and restricted cash are currently expected to be sufficient to fund our current operating plans into 2022. This cash runway guidance is based on our current operational plans and excludes any additional funding that may be received and business development activities that may be undertaken. In addition, our operating plans may change as a result of many factors currently unknown to us, including the short- and long-term effects of the COVID-19 pandemic, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, third-party funding, and marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or a combination of these approaches. In any event, we will require additional capital to pursue preclinical and clinical activities, regulatory approval and the commercialization of our current and future product candidates. Even if we believe we have sufficient capital for our current operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations. If we elect to do so, additional capital may not be available to us on acceptable terms, if at all. Our ability to access additional capital, and as a result our operating results and liquidity needs, could be negatively affected by market fluctuations and economic downturn. The COVID-19 pandemic has already resulted in a significant disruption of global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital, which could negatively affect our business. Any additional capital raising efforts may divert our management from its day-to-day activities, which may adversely affect our ability to develop and commercialize our current and future product candidates.

***Raising additional capital by issuing equity or debt securities may cause dilution to our existing stockholders, and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights.***

Until such time as we can generate substantial revenue from product sales, if ever, we expect to finance our cash needs through a combination of equity and debt financings, strategic alliances and license and development agreements in connection with any future collaborations. To the extent that we raise additional capital by issuing equity securities, our existing stockholders’ ownership may experience substantial dilution, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a stockholder. Equity and debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as redeeming our shares, making investments, incurring additional debt, making capital expenditures or declaring dividends.

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The incurrence of indebtedness could result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants therein, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely affect our ability to conduct our business.

If we raise additional capital through collaborations, strategic alliances or third-party licensing arrangements, we may have to relinquish valuable rights to our intellectual property, future revenue streams, research programs or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise additional capital through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise develop and market ourselves.

***We may be required to make payments under licenses applicable to nevanimibe and MLE-301.***

We have certain milestone and royalty payments related to nevanimibe and MLE-301. We acquired worldwide, exclusive rights to nevanimibe pursuant to our license agreement with the Regents of the University of Michigan (the "University of Michigan"), entered into in June 2013 (the "UM License Agreement"). Under the terms of the UM License Agreement, we are obligated to make significant milestone and royalty payments in connection with the attainment of certain development steps and the sale of resulting products with respect to nevanimibe, as well as other material obligations. However, due to our decision to cease investing in the nevanimibe program, we do not expect to achieve the development and commercialization milestones that would trigger such payments.

We acquired worldwide, exclusive rights to MLE-301 pursuant to a license agreement with F. Hoffmann-La Roche Ltd and Hoffman-La Roche Inc. (collectively, "Roche," and such agreement, the "Roche License Agreement."). Under the terms of the Roche License Agreement, we are obligated to make significant milestone and royalty payments in connection with the attainment of certain development steps and the sale of resulting products with respect to MLE-301, as well as other material obligations. While we do not expect to incur significant milestone or other non-royalty obligations under the Roche License Agreement in the near term, if milestone or other non-royalty obligations become due, we may not have sufficient funds available to meet our obligations, which will materially adversely affect our business operations and financial condition.

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

Because we have limited financial and managerial resources, we focus on research programs and product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with respect to our own product candidates for additional indications and other product candidates or diseases that later prove to have greater commercial potential. Our resource allocation decisions may ultimately not result in successful clinical development programs and may cause us to fail to capitalize on other viable product candidates, commercial products or profitable market opportunities. In addition, our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. For example, we discontinued the development of livoletide as a potential treatment of patients with PWS after receiving results from the Phase 2b trial in April 2020, and we do not currently plan further investment in the development of nevanimibe for the treatment of CAH based on the interim data review of the Phase 2b trial completed in June 2020.

Further, if we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through sale, collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to retain sole development and commercialization rights, which could materially adversely affect our business operations and financial condition.

**Risks Related to Development and Commercialization**

***Our future success is dependent on the successful clinical development, regulatory approval and subsequent commercialization of MLE-301 and any future product candidates. If we are not able to obtain the required regulatory approvals, we will not be able to commercialize our current or future product candidates and our ability to generate revenue will be adversely affected.***

We do not have any drugs that have received regulatory approval and may never be able to develop marketable product candidates. An inability to effectively commercialize our product candidates and to maximize their potential where possible through successful research and development activities, whether due to the impacts of the ongoing COVID-19 pandemic or otherwise, could have an adverse effect on our business, financial condition, results of operations and growth prospects.

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We expect that a substantial portion of our efforts and expenses for the foreseeable future will be devoted to the clinical development of MLE-301 and other future product candidates and, as a result, our business currently depends heavily on the successful development, regulatory approval and commercialization of MLE-301 and other future product candidates. We cannot be certain that MLE-301 or any other future product candidate will receive regulatory approval or be successfully commercialized even if we receive regulatory approval. The research, testing, manufacturing, safety, efficacy, labeling, approval, sale, marketing and distribution of our product candidates are, and will remain, subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and similar foreign regulatory authorities. Failure to obtain regulatory approval for MLE-301 or any other future product candidate in the United States or other jurisdictions will prevent us from commercializing and marketing MLE-301 or any other future product candidate.

Even if we were to successfully obtain approval from the FDA and comparable foreign regulatory authorities for our product candidates, any approval might contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval for our product candidates, or any approval contains significant limitations, on our own or with any future collaborators, we may not be able to obtain sufficient funding or generate sufficient revenue to continue the development of any other product candidate that we may in-license, develop or acquire in the future.

Furthermore, even if we obtain regulatory approval for MLE-301 or any other future product candidate, we will still need to develop a commercial infrastructure, or otherwise develop relationships with collaborators to commercialize, establish a commercially viable pricing structure and obtain approval for adequate reimbursement from third-party and government payors. If we, or our collaborators, are unable to successfully commercialize MLE-301 or any other future product candidate, we may not be able to generate sufficient revenue to continue our business.

***Preclinical studies or earlier clinical trials are not necessarily predictive of future results and the results of our clinical trials may not support our MLE-301 claims.***

We initiated a Phase 1 study of MLE-301 in September 2020, however MLE-301 will require further clinical testing before we are prepared to submit an NDA or other similar application for regulatory approval. We cannot predict with any certainty if or when we might apply for regulatory approval for MLE-301 for the treatment of VMS or whether any such application will be approved by the relevant regulatory authority. Human clinical trials are very expensive and difficult to design and implement, in part because they are subject to rigorous regulatory requirements. For instance, the FDA or foreign regulatory authorities may not agree with our proposed endpoints for any clinical trials of MLE-301, even if validated in prior clinical trials of similar product candidates, which may delay the commencement of our future clinical trials. The FDA or foreign regulatory authorities may also not agree with our proposed trial designs or dosing regimens, which may likewise prevent or delay the commencement of our future clinical trials. The clinical trial process is also time-consuming. We estimate that clinical trials of MLE-301 for the treatment of VMS will take the next several years to complete.

Success in preclinical testing and early clinical trials does not ensure that later and/or pivotal clinical trials will generate the same results, or otherwise provide adequate data to demonstrate the safety and efficacy of a product candidate. Frequently, product candidates that have shown promising results in early clinical trials have subsequently suffered significant setbacks in later or pivotal clinical trials. Failure can occur at any stage of the trials, and we could encounter problems that cause us to abandon or repeat clinical trials. For example, we expended substantial time and resources on a previous product candidate, MLE4901, an NK3R antagonist, which we ceased developing in 2017 due to concerns relating to elevated liver enzymes observed in clinical trials. Similarly, at least one of our competitors has publicly announced similar concerns in one of their clinical trials of an NK3R antagonists; however, they have indicated that they are continuing development of that product candidate with revised dosing for their Phase 3 trial. We are currently developing MLE-301, an NK3R antagonist, and there can be no assurance that we will not face similar development challenges with regard to this product candidate. We also discontinued our development of livoletide as a potential treatment of patients with PWS after receiving results from the Phase 2b trial in April 2020. In addition, we do not currently plan to make further investment in the development of nevanimibe for the treatment of CAH, based on the interim data review of the Phase 2b trial completed in June 2020. Further, we may encounter challenges in the clinical development of product candidates for reasons unrelated to the observed safety or efficacy of such product candidates in prior clinical trials. For example, in our Phase 2 clinical trial for the treatment of Cushing’s syndrome ("CS"), we experienced slower than anticipated enrollment, which could have made impractical further development of nevanimibe for the treatment of CS. As a result of the difficulty in enrolling this trial, we discontinued this Phase 2 clinical trial in August 2019 and discontinued development of nevanimibe for the treatment of CS. In addition, because we may at times pursue the treatment of multiple indications for a single product candidate, setbacks or failures in, or termination of, clinical development for one indication may have a negative impact on the clinical development for the treatment of other indications.

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Our approach to targeting endocrine diseases where current therapies do not exist or are insufficient, is novel and unproven, and as such, the cost and time needed to develop MLE-301 or any future product candidate is difficult to predict and our efforts may not be successful. If we do not observe favorable results in future or planned clinical trials of MLE-301, we may decide to delay or abandon development of MLE-301, which could harm our business, financial condition and results of operations. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials.

***We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.***

Before obtaining marketing approval from regulatory authorities for the sale of MLE-301 and any future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidate for its intended indication. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

•direct and indirect effects of the ongoing COVID-19 pandemic on various aspects and stages of the clinical development process;

•significant reprioritization and diversion of healthcare resources away from the conduct of clinical trials as a result of the ongoing COVID-19 pandemic, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;

•failure to obtain regulatory approval to commence a trial;

•unforeseen safety issues;

•determination of dosing issues;

•lack of effectiveness during clinical trials;

***•***inability to reach agreement on acceptable terms with prospective contract research organizations (“CROs”), and clinical trial sites;

•slower than expected rates of patient recruitment, failure to recruit adequate numbers of suitable patients to participate in our clinical trials or failure to maintain participation of recruited patients in clinical trials;

•interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel, quarantines or social distancing protocols imposed or recommended by federal or state governments, employers and others in connection with the ongoing COVID-19 pandemic;

•failure to manufacture sufficient quantities of a product candidate for use in clinical trials;

•inability to monitor patients adequately during or after treatment; and

•inability or unwillingness of medical investigators to follow our clinical protocols.

Further, we, the FDA, an institutional review board, or other regulatory authority may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including, for example, the FDA’s good clinical practice (“GCP”), regulations, that we are exposing participants to unacceptable health risks, or if the FDA or other regulatory authority, as the case may be, finds deficiencies in our IND application or other submissions, or the manner in which the clinical trials are conducted. Therefore, we cannot predict with any certainty the schedule for commencement and completion of future clinical trials. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our current and future product candidates could be harmed, and our ability to generate revenue from our current or future product candidates, once approved, may be delayed or eliminated. In addition, any delays in our clinical trials could increase our costs, slow down the approval process and jeopardize our ability to commence product sales and generate revenue. Any of these occurrences may harm our business, financial condition and results of operations. In addition, many of the factors that cause, or lead to, a delay in the

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commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

Moreover, principal investigators for our clinical trials may serve as our scientific advisors or consultants from time to time and receive compensation in connection with such services. We will be required to report these relationships to the FDA or other regulatory authorities as part of the drug approval process. The FDA or other regulatory authorities may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial results. They may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or other regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

Due to the COVID-19 pandemic, we experienced a disruption in our ability to enroll and assess patients in our Phase 2b study of nevanimibe for the treatment of patients with CAH, which we discontinued after an interim data review from the Phase 2b study completed in June 2020. For our trials of MLE-301, including the recently initiated Phase 1 clinical trial of MLE-301, or future product candidates, we may in the future experience similar or other disruptions or delays in our ability to initiate trial sites, enroll and assess patients, maintain patient enrollment, acquire supplies of study drug, report trial results, or interact with regulators, ethics committees or other important agencies due to limitations in employee resources or otherwise. In addition, some patients may not be able to comply with clinical trial protocols if quarantines or shelter-in-place orders impede patient movement or interrupt healthcare services. Similarly, our ability to recruit and retain patients and principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19 and adversely impact our clinical trial operations. In light of the ongoing COVID-19 pandemic, we have taken measures in past clinical trials to implement remote and virtual approaches to clinical development, including remote patient monitoring and home delivery of drug treatments where possible, and if the COVID-19 pandemic continues and persists for an extended period of time, we could experience significant disruptions to our clinical development timelines, which would adversely affect our business, financial condition, results of operations and growth prospects.

***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 our current product candidates or any future product candidates that we may develop.***

We have never obtained marketing approval for a product candidate. It is possible that the FDA may refuse to accept for substantive review any NDAs that we submit for our product candidates or may conclude after review of our data that our application is insufficient to obtain marketing approval of our product candidates. If the FDA does not accept or approve our NDAs for any of our product candidates, it may require that we conduct additional clinical trials, preclinical studies or manufacturing validation studies and submit that data before it will reconsider our applications. Depending on the extent of these or any other FDA-required trials or studies, approval of any NDA or application that we submit may be delayed by several years or may require us to expend more resources than we have available. It is also possible that additional trials or studies, if performed and completed, may not be considered sufficient by the FDA to approve our NDAs.

Any delay in obtaining, or an inability to obtain, marketing approvals would prevent us from commercializing our product candidates, generating revenues and achieving and sustaining profitability. If any of these outcomes occurs, we may be forced to abandon our development efforts for our product candidates, which could significantly harm our business, prospects, operating results and financial condition.

***Enrollment and retention of patients in clinical trials is a competitive, expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside our control.***

Identifying and qualifying patients to participate in our clinical trials is critical to our success. We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials, and even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. Patient enrollment and retention in clinical trials, including our ongoing clinical trial for MLE-301, depends on many factors, including: the size of the patient population, the nature of the trial protocol, our ability to recruit clinical trial investigators with the appropriate competencies and experience, the existing body of safety and efficacy data with respect to the study drug, the number and nature of competing treatments and ongoing clinical trials of competing drugs for the same disease, the proximity of patients to clinical sites and the eligibility criteria for the trials, our ability to obtain and maintain patient consents and the risk that patients enrolled in clinical trials will drop out of the trials before completion.

Patient enrollment may also be affected by the ongoing COVID-19 pandemic due to the prioritization of hospitalization resources toward this pandemic, exposure of healthcare providers to COVID-19 and difficulties for patients to access clinical

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trial sites and comply with clinical trial protocols. For example, we initiated a Phase 1 study of MLE-301 in September 2020. While we have taken measures to implement remote and virtual approaches to clinical development in prior clinical trials, including remote patient monitoring and home delivery of drug treatments where possible, we cannot at this time fully forecast the scope of impacts that the COVID-19 pandemic may have on our ability to initiate trial sites, enroll and assess patients, acquire supplies of study drug and report trial results.

The competitive nature of clinical trials in the pharmaceutical and biotechnology industries may make it difficult for us to recruit a sufficient number of patients to complete any of our clinical trials or may increase costs. We may not be able to initiate or continue to support clinical trials of our product candidates for one or more indications, or any future product candidates, if we are unable to locate and enroll a sufficient number of eligible participants in these trials as required by the FDA or other regulatory authorities. For example, in our Phase 2 clinical trial for CS we experienced slower than anticipated enrollment, which could have made impractical further development of nevanimibe for the treatment of CS. As a result of the difficulty in enrolling this trial, we elected to discontinue this Phase 2 clinical trial in August 2019 and discontinued development of nevanimibe for the treatment of CS. Even if we are able to enroll a sufficient number of patients in our clinical trials, if the pace of enrollment is slower than we expect, the development costs for our product candidates may increase and the completion of our trials may be delayed or our trials could become too expensive or impractical to complete.

Furthermore, any negative results we may report in clinical trials of our product candidates may make it difficult or impossible to recruit and retain patients in other clinical trials of those product candidates. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop MLE-301 or any other product candidate or could render further development impossible. For example, before we ceased investment in the nevanimibe program, we paused enrollment in the Phase 2b trial of nevanimibe for patients with CAH as a result of the COVID-19 pandemic. In addition, we may rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials and, while we have and would in the future intend to enter into agreements governing their services, we will be limited in our ability to compel their actual performance.

***Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.***

During the conduct of clinical trials, clinical investigators monitor changes in patients’ health, including illnesses, injuries and discomforts. Often, it is not possible to determine whether or not the product candidate being investigated caused these conditions, and regulatory authorities may draw different conclusions or require additional testing to confirm these determinations if they occur. In addition, it is possible that as we test MLE-301 or any other product candidate in larger, longer and more extensive clinical programs, or as use of any current or future product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials, will be observed or reported by subjects. If clinical testing indicates that MLE-301 or any future product candidate has side effects or causes serious or life-threatening side effects, we may need to change the design of ongoing clinical trials or adjust dosing levels in ongoing or future clinical trials, and the development of the product candidate may be delayed or terminated entirely. Further, if the product candidate has received regulatory approval, such approval may be revoked, which would materially harm our business, prospects, operating results and financial condition.

Moreover, if we elect or are required to modify, delay, suspend or terminate any clinical trial for our product candidates, the commercial prospects of our product candidates may be harmed and our ability to generate revenue through their sale may be delayed or eliminated. Any of these occurrences may harm our business, financial condition and prospects significantly.

***We face substantial competition, and our operating results will suffer if we fail to compete effectively.***

The commercialization of new drugs is competitive, and we may face worldwide competition from major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies and ultimately generic companies. Our competitors may develop or market therapies that are more effective, safer or less costly than any that we are commercializing, or may obtain regulatory or reimbursement approval for their therapies more rapidly than we may obtain approval for ours.

We are aware of a number of other clinical stage companies that are working to develop drugs that would compete, directly or indirectly, against MLE-301 for the treatment of VMS.

Astellas is developing fezolinetant and is conducting their Phase 3 VMS program. Bayer Pharmaceuticals recently purchased KaNDy Therapeutics and with it the dual NK1/3 receptor antagonist, NT-814. Bayer has announced that Phase 3 clinical trials

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of NT-814 will begin in 2021. Sojournix is currently studying SJX-653 in a Phase 2 trial. Additionally, Acer Therapeutics has announced their intentions to develop osanetant, an NK3R antagonist, for induced vasomotor symptoms stemming from hormonal therapies used to treat breast and prostate cancer, but have not announced definite plans for developing this product candidate for menopausal vasomotor symptoms.

Many of our existing or potential competitors may have substantially greater financial, technical and human resources than we do, and significantly greater experience in the discovery and development of product candidates, including in the recruitment of patients for clinical trials, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries. Our current and potential future competitors may also have significantly more experience commercializing drugs that have been approved for marketing. If we are not able to compete effectively against existing and potential competitors, our business and financial condition may be harmed.

Mergers and acquisitions in the pharmaceutical and biotechnology industries could result in even more resources being concentrated among a small number of our competitors. Competition may reduce the number and types of patients available to us to participate in clinical trials, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors.

Competition may further increase as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing, on an exclusive basis, drugs that are more effective or less costly than any product candidate that we may develop.

Any inability to successfully complete clinical development of a product candidate could result in additional costs or impair or eliminate our ability to generate revenue from future sales of such product candidate, if approved, or from any regulatory and commercialization milestone with respect to such product candidate. In addition, if we make manufacturing or formulation changes to MLE-301 or any future product candidates, we may need to conduct additional testing to bridge our modified product candidates to earlier versions. Clinical trial delays, including in the recently initiated Phase 1 clinical trial of MLE-301, could also shorten any periods during which we may have the exclusive right to commercialize MLE-301 or any future product candidates, or allow our competitors to bring comparable drugs to market before we do, which could impair our ability to successfully commercialize MLE-301 or any future product candidates, and may harm our business, financial condition and results of operations.

Established pharmaceutical and biotechnology companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make MLE-301 or any future product candidate less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing and receiving FDA or other regulatory authority approval, or commercializing drugs before we do, which would have an adverse impact on our business and results of operations.

The availability of our competitors’ products could limit the demand and the price we are able to charge for any product candidate we develop. The inability to compete with existing or subsequently introduced drugs would harm our business, prospects, financial condition and results of operations.

***The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming and inherently unpredictable, and even if we obtain approval for a product candidate in one country or jurisdiction, we may never obtain approval for, or commercialize, that product candidate in any other jurisdiction, which would limit our ability to realize our full market potential.***

Prior to obtaining approval to commercialize a product candidate in any jurisdiction, we or our collaborators must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA or foreign regulatory agencies, that such product candidates are safe and effective for their intended uses. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the FDA or foreign regulatory agencies may believe the clinical trials do not show the appropriate balance of safety and efficacy in the indication being sought or may interpret the data differently than we do, and deem the results insufficient to demonstrate the appropriate balance of safety and efficacy at the level required for product approval. Further, the regulatory authorities may not complete their review processes in a timely manner, or we may otherwise not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future

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legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials and the review process.

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

The FDA or any foreign regulatory bodies can delay, limit or deny approval of our product candidates or require us to conduct additional preclinical or clinical testing or abandon a program for many reasons, including:

•the FDA or the applicable foreign regulatory agency’s disagreement with the design or implementation of our clinical trials;

•negative or ambiguous results from our clinical trials or results that may not meet the level of statistical significance required by the FDA or comparable foreign regulatory agencies for approval;

•serious and unexpected drug-related side effects experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;

•our inability to demonstrate to the satisfaction of the FDA or the applicable foreign regulatory body that our product candidates are safe and effective for the proposed indication;

•the FDA’s or the applicable foreign regulatory agency’s disagreement with the interpretation of data from preclinical studies or clinical trials;

•our inability to demonstrate the clinical and other benefits of our product candidates outweigh any safety or other perceived risks;

•the FDA’s or the applicable foreign regulatory agency’s requirement for additional preclinical studies or clinical trials;

•the FDA’s or the applicable foreign regulatory agency’s disagreement regarding the formulation, labeling or the specifications of our product candidates;

•the FDA’s or the applicable foreign regulatory agency’s failure to approve the manufacturing processes or facilities of third-party manufacturers with which we contract, including failure of such manufacturers to pass the required pre-approval inspections; or

•the potential for approval policies or regulations of the FDA or the applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Even if we eventually complete clinical testing and receive approval of an NDA or foreign marketing application for our product candidates, the FDA or the applicable foreign regulatory agency may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, or the implementation of a Risk Evaluation and Mitigation Strategy (“REMS”), which may be required to ensure safe use of the drug after approval. The FDA or the applicable foreign regulatory agency also may approve a product candidate for a more limited indication or patient population than we originally requested, and the FDA or applicable foreign regulatory agency may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and would negatively impact our business and results of operations.

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***If we are not able to obtain orphan drug designations or exclusivity for any of our current or future product candidates for which we seek such designation, the potential profitability of any such product candidates could be limited.***

Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product as an orphan drug if the treatment is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for a disease for which it receives the designation, then the product is entitled to a period of marketing exclusivity that precludes the applicable regulatory authority from approving another marketing application for the same product for the same disease for the exclusivity period except in limited situations. For purposes of small molecule drugs, the FDA defines “same drug” as a drug that contains the same active moiety and is intended for the same use as the drug in question.

We have received orphan drug designation from the FDA and the EMA for previous product candidates, and we may seek orphan drug designation, where applicable, for our current product candidates in additional indications or for our future product candidates. However, obtaining an orphan drug designation can be difficult and we may not be successful in doing so for any of our current or future product candidates, in any applicable indication. Even if we were to obtain orphan drug designation for a product candidate, we may not obtain orphan exclusivity and that exclusivity may not effectively protect the product candidate from the competition of different products or drugs for the same condition, which could be approved during the exclusivity period. Additionally, after an orphan drug is approved, the FDA could subsequently approve another application for the same product for the same disease if the FDA concludes that the later product is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusive marketing rights in the United States also may be lost if the FDA later determines that the request for designation was materially defective, the prevalence of the orphan disease is found to increase such that the qualifying criterion is no longer met 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. The failure to obtain an orphan drug designation for any product candidates we may develop and seek it for, the inability to maintain that designation for the duration of the applicable period, or the inability to obtain or maintain orphan drug exclusivity could reduce our ability to make sufficient sales of the applicable product candidates to balance our expenses incurred to develop it, which would have a negative impact on our operational results and financial condition.

***If we are not able to obtain required regulatory approvals, we will not be able to commercialize MLE-301 or any future product candidate and our ability to generate revenue will be harmed.***

MLE-301 and any future product candidate, and the activities associated with their development and commercialization, including their design, research, testing, manufacture, safety, efficacy, recordkeeping, labeling, packaging, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by similar regulatory authorities outside the United States. Failure to obtain marketing approval for MLE-301 and any future product candidate or failure to meet post-marketing requirements will prevent us from commercializing MLE-301 and any future product candidate.

We have not yet received approval from regulatory authorities to market any product candidate in any jurisdiction, and it is possible that MLE-301 or any future product candidates will never obtain the appropriate regulatory approvals necessary for us to commence product sales. Neither we nor any future collaborator is permitted to market any of our product candidates in the United States until we receive regulatory approval of an NDA from the FDA.

The time required to obtain approval of an NDA 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. Prior to submitting an NDA to the FDA or an equivalent application to other foreign regulatory authorities for approval of MLE-301 for the treatment of VMS, we will need to complete its currently planned registration clinical trials and any additional trials that the FDA may require us to complete.

Due to the ongoing COVID-19 pandemic, it is possible that we could experience delays in the timing of our interactions with regulatory authorities due to absenteeism by governmental employees, inability to conduct planned physical inspections related to regulatory approval, or the diversion of regulatory authority efforts and attention to approval of other therapeutics or other activities related to COVID-19, which could delay anticipated approval decisions and otherwise delay or limit our ability to make planned regulatory submissions or obtain new product approvals. Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with MLE-301, we may:

•be delayed in obtaining marketing approval for MLE-301, if at all;

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•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 additional post-marketing testing requirements;

•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 drug or impose restrictions on its distribution in the form of REMS;

•be subject to the addition of labeling statements, such as warnings or contraindications;

•be sued; or

•experience damage to our reputation.

Furthermore, 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 may rely on third-party CROs and consultants to assist us in filing and supporting the applications necessary to gain marketing approvals. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each disease to establish the safety and efficacy of MLE-301 and any future product candidate for that disease. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities.

***Even if we obtain regulatory approval for MLE-301 or future product candidates, we will remain subject to ongoing regulatory oversight.***

Even if we obtain any regulatory approval for MLE-301 or future product candidates, the approved product will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. For example, we must comply with the FDA’s advertising and promotion requirements, such as those related to direct-to-consumer advertising and the prohibition on promoting products for uses or in patient populations that are not described in the product’s approved labeling. In addition, any regulatory approvals that we receive for MLE-301 or future product candidates may also be subject to REMS limitations on the approved indicated uses for which the drug may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the quality, safety and efficacy of the drug.

In addition, drug manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the NDA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a drug, such as adverse events of unanticipated severity or frequency, or problems with the facility where the drug is manufactured or disagrees with the promotion, marketing or labeling of that drug, a regulatory authority may impose restrictions relative to that drug, the manufacturing facility or us, including requiring recall or withdrawal of the drug from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of MLE-301 or future product candidates, a regulatory authority may, among other things:

•issue a warning letter asserting that we are in violation of the law;

•seek an injunction or impose administrative, civil or criminal penalties or monetary fines;

•suspend or withdraw regulatory approval;

•suspend any ongoing clinical trials;

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•refuse to approve a pending NDA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;

•restrict the marketing or manufacturing of the drug;

•seize or detain the drug or otherwise require the withdrawal of the drug from the market;

•refuse to permit the import or export of product candidates; or

•refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize MLE-301 or future product candidates, and harm our business, financial condition and results of operations.

In addition, the FDA’s policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could suspend or restrict regulatory approval of MLE-301 or future product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would harm our business, financial condition and results of operations.

***Even if one of our product candidates receives marketing approval, it may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.***

Even if one of our product candidates receives marketing approval, it may fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If any such product candidate does 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 a product candidate, if approved for commercial sale, will depend on a number of factors, including but not limited to:

•the efficacy and potential advantages compared to alternative treatments;

•the success of our efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits of our products;

•effectiveness of sales and marketing efforts;

•the cost of treatment in relation to alternative treatments, including any similar generic treatments;

•our ability to offer our drugs, once approved, for sale at competitive prices;

•the convenience and ease of administration compared to alternative treatments;

•the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;

•the strength of marketing and distribution support;

•the availability of third-party coverage and adequate reimbursement, and patients’ willingness to pay out-of-pocket in the absence third-party coverage or adequate reimbursement;

•the prevalence and severity of any side effects; and

•any restrictions on the use of our drugs, once approved, together with other medications.

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***If the market opportunities for our product candidates are smaller than we believe they are, our product revenues may be adversely affected and our business may suffer.***

Our efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits of our products, if and when approved, may require significant resources and may never be successful. If the actual number of patients is smaller than we estimate for any disease that we are targeting, if we cannot raise awareness of these diseases and diagnosis is not improved, or if we are not able to find market acceptance for our products in comparison to competitive products, our revenue and ability to achieve profitability may be adversely affected. Because we expect sales of MLE-301, if approved, to generate substantially all of our product revenue for the foreseeable future, the failure of MLE-301 to find market acceptance would harm our business.

***If we are unable to establish sales, marketing and distribution capabilities, either on our own or in collaboration with third-parties, we may not be successful in commercializing our product candidates, if approved.***

We do not have any infrastructure for the sales, marketing or distribution of our products, and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market any product that may be approved, we must build our sales, distribution, marketing, managerial and other non-technical capabilities, or make arrangements with third-parties to perform these services. There can be no assurance we will be able to do so in a cost-effective manner, on terms favorable to us, or at all.

In the event we seek to establish our own sales, marketing and distribution capabilities, there will be significant expenses and risks involved, including with our ability to hire, retain and appropriately incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. These expenses and risks will be more significant in the event we seek to commercialize product candidates targeting larger indications, such as MLE-301, on our own. Any failure or delay in the development of our internal sales, marketing and distribution capabilities could delay any product launch, which would adversely impact its commercialization.

Factors that may inhibit our future efforts to commercialize our products on our own include:

•our inability to raise additional capital through equity or debt financings or through lending and licensing arrangements;

•our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;

•the inability of sales personnel to obtain access to educate adequate numbers of physicians as to the benefits or our drug products;

•the inability of reimbursement professionals to negotiate arrangements, for formulary access, reimbursement, and other acceptance by payors;

•restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;

•the lack of complementary medicines to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and

•unforeseen costs and expenses associated with creating an independent sales and marketing organization.

Further, we do not anticipate having the resources in the foreseeable future to allocate to the sales and marketing of our product candidates in certain markets overseas. Therefore, our future success will depend, in part, on our ability to enter into and maintain collaborative relationships for such capabilities, the collaborator’s strategic interest in a product and such collaborator’s ability to successfully market and sell the product. We intend to pursue collaborative arrangements regarding the sale and marketing of our product candidates, if approved, for certain markets overseas; however, we cannot assure you that we will be able to establish or maintain such collaborative arrangements, or if able to do so, that we will have effective sales forces. To the extent that we depend on third-parties for marketing and distribution, any revenue we receive will depend upon the efforts of such third-parties, and there can be no assurance that such efforts will be successful.

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If we are unable to negotiate a collaborative relationship for the commercialization of our product candidates, we may be forced to delay our potential commercialization or reduce the scope of our sales or marketing activities for them. If we elect to increase our expenditures to fund commercialization activities itself, we will 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 will not be able to bring our product candidates to market or generate product revenue. We could enter into arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be ideal and we may be required to relinquish rights to our product candidates or otherwise agree to terms unfavorable to us, any of which may have an adverse effect on our business and results of operations.

If we are unable to establish adequate sales, marketing and distribution capabilities, either on our own or in collaboration with third-parties, we will not be successful in commercializing our product candidates and may not become profitable. We will be competing with many companies that currently have extensive and well-funded sales and marketing operations. Without an internal team or the support of a third-party to perform sales and marketing functions, we may be unable to compete successfully against these more established companies.

***If approved, our product candidates will face competition from less expensive generic products of competitors, and, if we are unable to differentiate the benefits of our product candidates over these less expensive alternatives, we may never generate meaningful product revenues.***

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

***Even if we obtain and maintain approval for our current and future product candidates from the FDA, we may nevertheless be unable to obtain approval for our product candidates outside of the United States, which would limit our market opportunities and could harm our business.***

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. If approved, sales of MLE-301 and any future product candidate outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for any product candidates, if approved, is also subject to approval. Obtaining approval for MLE-301 or any future product candidate in the European Union from the European Commission following the opinion of the EMA, if we choose to submit a marketing authorization application there, would be a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the drug may be marketed, require extensive warnings on the drug labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of MLE-301 or any future product candidate in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for MLE-301 or any future product candidate may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of MLE-301 or any future product candidate will be negatively impacted, and our business, prospects, financial condition and results of operations could be harmed.

***We are exposed to a variety of risks associated with our international operations.***

Since the closing date of the merger we completed with OvaScience in 2018 (“the Merger”), we have been engaged in the process of winding up various subsidiaries of OvaScience, some or all of which are in foreign jurisdictions. We expect to incur

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additional costs to complete this process. Moreover, even if we successfully wind up these entities, we may be exposed to liability in these foreign jurisdictions as a result of their historical operations.

In addition, in December 2017, we acquired Alizé Pharma SAS (“Alizé”), a biopharmaceutical company based in Lyon, France. As of October 1, 2020, we had 22 employees located in the United States and 2 employees located in France. Our past and current global operations expose us to numerous and sometimes conflicting legal, tax and regulatory requirements, and violations or unfavorable interpretation by the respective authorities of these regulations could harm our business. Risks associated with international operations include the following, and these risks may be more pronounced if we seek to commercialize MLE-301 or any future product candidates outside of the United States:

•different regulatory requirements for approval of therapies 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 particular foreign economies and markets;

•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;

•foreign reimbursement, pricing and insurance regimes;

•workforce uncertainty in countries where labor unrest is more common than in the United States;

•changes in diplomatic and trade relationships;

•anti-corruption laws, including the FCPA, and its equivalent in foreign jurisdictions, such as the UK Bribery Act;

•production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and

•business interruptions resulting from pandemics and public health emergencies, including those related to the COVID-19 pandemic, geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires.

In addition, there are complex regulatory, tax, labor, and other legal requirements imposed by both the European Union and many of the individual countries in and outside of Europe, with which we may need to comply. Many biopharmaceutical companies have found the process of marketing their own products in foreign countries to be very challenging.

Furthermore, in some countries, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product candidate. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after coverage and reimbursement have been obtained. Reference pricing used by various countries and parallel distribution or arbitrage between low-priced and high-priced countries, can further reduce prices. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies, which is time-consuming and costly. If coverage and reimbursement of our product candidates are unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed.

***Legal, political and economic uncertainty surrounding the exit of the U.K., from the European Union, (“EU”), may be a source of instability in international markets, create significant currency fluctuations, adversely affect our operations in the U.K. and pose additional risks to our business, revenue, financial condition, and results of operations.***

On June 23, 2016, the U.K. held a referendum in which a majority of the eligible members of the electorate voted for the U.K. to leave the EU. The U.K.’s withdrawal from the EU is commonly referred to as Brexit. The U.K. formally left the EU on January 31, 2020, and is now in a transition period through December 31, 2020. The lack of clarity over which EU laws and

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regulations will continue to be implemented in the U.K. after Brexit (including financial laws and regulations, tax and free trade agreements, intellectual property rights, data protection laws, supply chain logistics, environmental, health and safety laws and regulations, immigration laws and employment laws) may negatively impact foreign direct investment in the U.K., increase costs, depress economic activity and restrict access to capital. The uncertainty concerning the U.K.’s legal, political and economic relationship with the EU after Brexit may be a source of instability in the international markets, create significant currency fluctuations, and/or otherwise adversely affect trading agreements or similar cross-border co-operation arrangements (whether economic, tax, fiscal, legal, regulatory or otherwise) beyond the date of Brexit.

These developments, or the perception that any of them could occur, have had, and may continue to have, a significant adverse effect on global economic conditions and the stability of global financial markets, and could significantly reduce global market liquidity and limit the ability of key market participants to operate in certain financial markets. In particular, it could also lead to a period of considerable uncertainty in relation to the U.K. financial and banking markets, as well as on the regulatory process in Europe. Asset valuations, currency exchange rates and credit ratings may also be subject to increased market volatility. The long-term effects of Brexit will depend on any agreements (or lack thereof) between the U.K. and the EU and, in particular, any arrangements for the U.K. to retain access to EU markets either during a transitional period or more permanently.

Such a withdrawal from the EU is unprecedented, and it is unclear how the U.K.’s access to the European single market for goods, capital, services and labor within the EU, or single market, and the wider commercial, legal and regulatory environment, will impact us. We may also face new regulatory costs and challenges that could have an adverse effect on our operations. Depending on the terms of the U.K.’s withdrawal from the EU, the U.K. could lose the benefits of global trade agreements negotiated by the EU on behalf of its members, which may result in increased trade barriers that could make our doing business in the U.K. more difficult. Furthermore, there are likely to be changes to the way in which marketing approvals are granted in the U.K., which could add time and expense to the process by which our product candidates receive and maintain regulatory approval in the U.K. and across the European Economic Area in future.

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

We face an inherent risk of product liability exposure related to the testing of our current and future product candidates, and may face an even greater risk if we commercialize any product candidate that we may develop. If we cannot successfully defend ourselves against claims that any such 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 candidate 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 candidate that it may develop;

•injury to our reputation and significant negative media attention; and

•increased marketing costs to attempt to overcome any injury to our reputation or negative media attention.

In addition, we face an inherent risk of product liability exposure related to OvaScience’s prior use of fertility treatments in humans. Product liability claims involving OvaScience’s activities may be brought for significant amounts because OvaScience’s potential fertility treatments involved mothers and children. For example, it is possible that we will be subject to product liability claims that assert that OvaScience’s potential fertility treatments have caused birth defects in children or that such defects are inheritable. These claims could be made many years into the future based on effects that were not observed or observable at the time of birth. If we cannot successfully defend against claims that OvaScience’s potential fertility treatments caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in, among other things, significant costs to defend the related litigation; substantial monetary awards or payments to trial participants or patients; loss of revenue; and the diversion of management’s resources.

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Although we maintain product liability insurance coverage, such insurance 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.

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

OvaScience was 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. OvaScience’s prior operations involved the use of hazardous and flammable materials, including chemicals and biological materials. OvaScience’s prior operations also produced hazardous waste products. OvaScience generally contracted with third-parties for the disposal of these materials and wastes. In the event of contamination or injury resulting from OvaScience’s use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with OvaScience’s storage or disposal of biological, hazardous or radioactive materials.

**Risks Related to Regulatory Compliance**

***Our current and future relationships with investigators, health care professionals, consultants, third-party payors and customers may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.***

Our operations may be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws and regulations, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims laws and Physician Payments Sunshine Act and regulations. These laws may constrain our current and future business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our products for which we obtain marketing approval. In addition, we may be subject to patient privacy laws by both the federal government and the states and other countries in which we conduct our business. The laws that will affect our operations include, but are not limited to:

•the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers, formulary managers, and others on the other hand. In addition, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively PPACA, amended the intent requirement of the federal Anti-Kickback Statute, establishing that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;

•federal civil and criminal false claims laws, including the federal civil False Claims Act, and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent. PPACA provides, and recent government cases against pharmaceutical and medical device manufacturers support the view, that federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the federal civil False Claims Act;

***•***the federal Health Insurance Portability and Accountability Act of 1996 (“HIPAA”), which created additional federal civil and criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);

***•***HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (“HITECH”), and their implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as

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health plans, health care clearinghouses and certain health care providers, known as covered entities, and their business associates, as well as their subcontractors, who create, use or disclose individually identifiable health information on their behalf;

•federal transparency laws, including the federal Physician Payments Sunshine Act, that require certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children’s Health Insurance Program, with specific exceptions, to report annually to CMS information related to: (i) payments or other “transfers of value” made to physicians, as defined by such law, and teaching hospitals and (ii) ownership and investment interests held by physicians and their immediate family members. Beginning in 2022, applicable manufacturers also will be required to report such information regarding its relationships with physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists and certified nurse midwives during the previous year;

•state and foreign law equivalents of each of the above federal laws, such as state anti-kickback, self-referral, and false claims laws which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements as well as submitting claims involving healthcare items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical manufacturers to comply with the industry’s voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government that otherwise restricts payments that may be made to healthcare providers; state laws that require pharmaceutical manufacturers to file reports with states regarding marketing information, such as the tracking and reporting of gifts, compensation and other remuneration and items of value provided to healthcare professionals and entities; state laws that require the reporting of information related to drug pricing; and state and local laws requiring the registration of pharmaceutical sales representatives; and

•state and foreign laws that 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 preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third-parties will comply with applicable healthcare laws and regulations will involve substantial costs. However, because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including significant administrative, civil and criminal penalties, damages, fines, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of noncompliance with these laws, exclusion from participation in government health care programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm and the curtailment or restructuring of our operations, any of which could harm our ability to operate our business and our results of operations. Similar sanctions and penalties, as well as imprisonment, also can be imposed upon executive officers and employees, including criminal sanctions against executive officers under the so-called “responsible corporate officer” doctrine, even in situations where the executive officer did not intend to violate the law and was unaware of any wrongdoing.

The risk of us being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and its provisions are open to a variety of interpretations. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management’s attention from the operation of our business. The shifting compliance environment and the need to build and maintain a robust and expandable system to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a healthcare company such as we may run afoul of one or more of the requirements.

***Coverage and adequate reimbursement may not be available for our current or future product candidates, which could make it difficult for us to sell them profitably, if approved.***

Market acceptance and sales of any product candidates that we commercialize, if approved, will depend in part on the extent to which coverage and reimbursement for these drugs and related treatments will be available from third-party payors, including government health administration authorities and private health insurers. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided for any product candidates that we develop will be made on a plan-by-plan basis. As a result, the coverage determination process is often a time-consuming and costly process that may require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained. One payor’s determination to provide coverage

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for a drug does not assure that other payors will also provide coverage, and adequate reimbursement, for the drug. Additionally, a third-party payor’s decision to provide coverage for a therapy does not imply that an adequate reimbursement rate will be approved. Each plan determines whether it will provide coverage for a therapy, what amount it will pay the manufacturer for the therapy, and on what tier of its formulary it will be placed. The position on a formulary generally determines the co-payment that a patient will need to make to obtain the therapy and can strongly influence the adoption of such therapy by patients and physicians. Patients who are prescribed treatments for their conditions and providers prescribing such services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our drugs unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our drugs.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that coverage and reimbursement will be available for any drug that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Inadequate coverage and reimbursement may impact the demand for, or the price of, any drug for which we obtain marketing approval. If coverage and reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize MLE-301 and any future product candidates that we develop.

Additionally, there have been a number of legislative and regulatory proposals to change the healthcare system in the United States and in some foreign jurisdictions that could affect our ability to sell any future product candidates profitably. These legislative and regulatory changes may negatively impact the coverage and available reimbursement for MLE-301 and any future product candidates we may commercialize, following approval, if obtained.

***Healthcare legislative reform measures may have a negative impact on our business and results of operations.***

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any product candidates for which we obtain marketing approval.

In March 2010, PPACA was passed, which substantially changed the way healthcare is financed by both the government and private insurers, and significantly impacts the U.S. pharmaceutical industry. PPACA, among other things: (i) addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; (ii) increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations; (iii) established annual fees and taxes on manufacturers of certain branded prescription drugs; (iv) expanded the availability of lower pricing under the 340B drug pricing program by adding new entities to the program; and (v) established a new Medicare Part D coverage gap discount program, in which manufacturers must, as of January 1, 2019, agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer’s outpatient drugs to be covered under Medicare Part D.

There remain judicial and Congressional challenges to certain aspects of PPACA, as well as efforts by the Trump administration to repeal or replace certain aspects of PPACA. Since January 2017, President Trump has signed several Executive Orders and other directives designed to delay the implementation of certain provisions of PPACA or otherwise circumvent some of the requirements for health insurance mandated by PPACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of PPACA. While Congress has not passed comprehensive repeal legislation, several bills affecting the implementation of certain taxes under PPACA have been signed into law. The Tax Cuts and Jobs Act of 2017 (“Tax Act”), included a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by PPACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the “individual mandate”. Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the PPACA-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 PPACA, effective January 1, 2019, to increase from 50 percent 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.” In December 2018, CMS published a new final rule permitting further collections and payments to and from certain PPACA qualified health plans and health insurance issuers under the PPACA risk adjustment program. On April 27, 2020, the United States Supreme Court reversed a Federal Circuit decision that previously upheld Congress' denial of $12 billion in "risk corridor" funding. On December 14, 2018, a Texas U.S. District Court Judge ruled that the PPACA is unconstitutional in its entirety because the “individual mandate” was repealed by Congress as part of the Tax Act. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the

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case back to the District Court to determine whether the remaining provisions of the PPACA are invalid as well. On March 2, 2020, the United States Supreme Court granted the petitions for writs of certiorari to review this case but it is unclear when a decision is expected to be made. It is also unclear how such litigation and other efforts to repeal and replace the PPACA will impact the PPACA. We continue to evaluate the potential impact of PPACA and its possible repeal or replacement on our business.

We expect that PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we are able to charge for any approved drug in the United States. For example, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drugs. At the federal level, the Trump 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. On March 10, 2020, the Trump administration sent “principles” for drug pricing to Congress, calling for legislation that would, among other things, cap Medicare Part D beneficiary out-of-pocket pharmacy expenses, provide an option to cap Medicare Part D beneficiary monthly out-of-pocket expenses, and place limits on pharmaceutical price increases. Moreover, the Trump administration previously released a “Blueprint” to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. The Department of Health and Human Services (“HHS”), has solicited feedback on some of these measures and has implemented others under its existing authority. On July 24, 2020, the Trump administration announced four executive orders related to prescription drug pricing that attempt to implement several of the administration's proposals, including a policy that would tie Medicare Pare B drug prices to international drug prices; one that directs HHS to finalize the Canadian drug importation proposed rule previously issued by HHS and makes other changes allowing for personal importation of drugs from Canada; one that directs HHS to finalize the rulemaking process on modifying the anti-kickback law safe harbors for discounts for plans, pharmacies, and pharmaceutical benefit managers; and one the reduces costs of insulin and epipens to patients of federally qualified health centers. Although a number of these and other measures may require additional authorization to become effective, Congress and the Trump administration have each indicated that it 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, such measures are designed to encourage importation from other countries and bulk purchasing. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our drugs. 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.

In addition, other legislative changes have been adopted since PPACA was enacted. These changes include aggregate reductions in Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, following passage of the Bipartisan Budget Act of 2018, among other legislative amendments, will remain in effect through 2030 unless additional Congressional action is taken. The Coronavirus Aid, Relief and Economic Security Act (“CARES Act”), which was signed into law in March 2020 and is designed to provide financial support and resources to individuals and businesses affected by the COVID-19 pandemic, 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, among other things, further 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 new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and, accordingly, our financial operations.

Additional changes that may affect our business include those governing enrollment in federal healthcare programs, reimbursement changes, rules regarding prescription drug benefits under the health insurance exchanges and fraud and abuse and enforcement. Continued implementation of PPACA and the passage of additional laws and regulations may result in the expansion of new programs such as Medicare payment for performance initiatives, and may impact existing government healthcare programs, such as by improving the physician quality reporting system and feedback program. For each state that does not choose to expand its Medicaid program, there likely will be fewer insured patients overall, which could impact the

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sales, business and financial condition of manufacturers of branded prescription drugs. Where patients receive insurance coverage under any of the new options made available through PPACA, the possibility exists that manufacturers may be required to pay Medicaid rebates on their resulting drug utilization, a decision that could impact manufacturer revenues.

Further, it is possible that additional governmental action may be taken in response to the COVID-19 pandemic. For example, on August 6, 2020, the Trump administration issued another executive order that 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 China. The order is meant to 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.

***Regulatory, legislative or self-regulatory/standard developments regarding privacy and data security matters could adversely affect our ability to conduct our business.***

We are subject to and affected by laws, rules, regulations and industry standards related to data privacy and security, and restrictions or technological requirements regarding the collection, use, storage, security, retention or transfer of data. In the United States, the rules and regulations to which we may be subject include federal laws and regulations enforced by the Federal Trade Commission, HHS, and state privacy, data security, and breach notification laws, as well as regulator enforcement positions and expectations. Internationally, governments and agencies have adopted and could in the future adopt, modify, apply or enforce additional laws, policies, regulations, and standards covering privacy and data security that may apply to our business. New regulation or legislative actions regarding data privacy and security (together with applicable industry standards) may increase our costs of doing business. In addition to privacy and data security regulations currently in force in the jurisdictions where we operate, the European Union General Data Protection Regulation (“GDPR”), went into effect in May 2018. The GDPR contains numerous requirements and changes from existing European Union (“EU”), law, including more robust obligations on data processors and data controllers and heavier documentation requirements for data protection compliance programs. Specifically, the GDPR will introduce numerous privacy-related changes for companies operating in the EU, including greater control over personal data-by-data subjects (e.g., the “right to be forgotten”), increased data portability for EU consumers, data breach notification requirements, and increased fines. In particular, under the GDPR, fines of up to €20 million or up to 4% of the annual global revenue of the noncompliant company, whichever is greater, could be imposed for violations of certain of the GDPR’s requirements. The GDPR requirements apply not only to third-party transactions, but also to transfers of information between us and our subsidiaries, including employee information. However, despite our ongoing efforts to bring our practices into compliance before the effective date of the GDPR, we may not be successful either due to various factors within our control, such as limited financial or human resources, or other factors outside our control. It is also possible that local data protection authorities may have different interpretations of the GDPR, leading to potential inconsistencies amongst various EU member states. Any failure or alleged failure (including as a result of deficiencies in our policies, procedures, or measures relating to privacy, data security, marketing, or communications) by us to comply with laws, regulations, policies, legal or contractual obligations, industry standards, or regulatory guidance relating to privacy or data security, may result in governmental investigations and enforcement actions, litigation, fines and penalties, additional regulatory oversight and reporting obligations or adverse publicity. Further, because of the work-from-home policies we implemented due to COVID-19, information that is normally protected, including company confidential information, may be less secure.

We expect that there will continue to be new proposed laws, regulations and industry standards relating to privacy and data protection in the United States, the European Union, and in other jurisdictions, and we cannot determine the impact such future laws, regulations and standards may have on our business. Future laws, regulations, standards and other obligations or any changed interpretation of existing laws or regulations could impair our ability to operate our business and negatively impact our results of operations.

**Risks Related to Our Intellectual Property**

***We rely on the availability of licenses for intellectual property from third-parties and these licenses may not be available to us on commercially reasonable terms, or at all.***

The UM License Agreement provides certain patent rights and proprietary technology from the University of Michigan that are important or necessary to any future development of nevanimibe. In addition, we rely upon the Roche License Agreement to certain patent rights and proprietary technology from Roche that are important or necessary to our ongoing development of MLE-301. As of September 30, 2020, with respect to nevanimibe patent rights, we owned two issued U.S. patents, two pending U.S. patent applications, and a number of patent applications in other jurisdictions, and we jointly owned, with the University of Michigan, three issued U.S. patents, one pending U.S. patent application, and a number of patents and patent applications in

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other jurisdictions. In addition, as of September 30, 2020, with respect to MLE-301 patent rights, we owned one pending Patent Cooperation Treaty application, and we exclusively licensed from Roche one issued U.S. patent and a number of patents and pending patent applications in other jurisdictions. There is no guarantee that any of the foregoing patent applications will result in issued patents, or that any current patents or patent applications, if issued, will include claims that are sufficiently broad to cover our product candidates or future products, or to provide meaningful protection from our competitors in all territories in which we may wish to develop or commercialize our products in the future. We will be able to protect our proprietary rights from unauthorized use by third-parties only to the extent they are covered by valid and enforceable patents or are effectively maintained as trade secrets within our organization. If third-parties disclose or misappropriate our proprietary rights, it may have a material adverse effect on our business.

The licenses granted under the UM License Agreement and Roche License Agreement, respectively, are revocable under certain circumstances including if we cease to do business, fail to make the payments due thereunder, commit a material breach of the agreement that is not cured within a certain time period after receiving written notice or fail to meet certain specified development and commercial timelines. In particular, due to our decision to cease investing in the nevanimibe program, we do not expect to meet the development and commercial timelines set forth in the UM License Agreement. As a result, we expect the licenses granted under the UM License Agreement to be revocable beginning January 1, 2021, unless the University of Michigan agrees to extend those timelines. In the event of any revocation of our licenses, our ability to out-license nevanimibe for a particular drug indication may be diminished.

Additionally, termination of the UM License Agreement or Roche License Agreement may result in us having to negotiate a new or reinstated agreement, which may not be available to us on equally favorable terms, or at all, which may mean, in the case of the Roche License Agreement, that we are unable to develop or commercialize MLE-301. Additionally, the UM License Agreement, Roche License Agreement and other licenses we may enter into in the future may not provide exclusive rights to use such intellectual property and technology at all, in all relevant fields of use and/or in all territories in which we may wish to develop or commercialize our product candidates in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products, including in territories included in the UM License Agreement and Roche License Agreement.

Licenses to additional third-party patents and materials that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could harm our business and financial condition.

***Our intellectual property licenses and agreements with third-parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.***

We currently depend, and will continue to depend, on the Roche License Agreement. If Roche terminates the Roche License Agreement due to a breach of any of our material obligations under the Roche License Agreement or due to our insolvency, or if we terminate the Roche License Agreement without cause, the rights and licenses granted by Roche to us under the Roche License Agreement will terminate on the effective date of the termination. In such event, if Roche provides us with timely notice, and to the extent reasonably requested by Roche, we must transfer to Roche all regulatory filings and approvals, all final preclinical, non-clinical and clinical study reports and clinical study protocols, trademarks, and all data, including clinical data, materials and information, in our possession and control related to MLE-301 necessary or reasonably useful for Roche to continue to develop and commercialize MLE-301. Further, if the effective date of such a termination is after the first commercial sale of the first MLE-301 product, Roche shall have a worldwide, non-exclusive, sublicensable, transferable license to research, develop, manufacture, and sell MLE-301 compounds and products. In such event, Roche would pay to us royalty fees with respect to the sale of those compounds and products. Further development and commercialization of MLE-301 may, and development of any future product candidates may, require us to enter into additional license, assignment or collaboration agreements. The agreements under which we currently hold or 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.

If any of our current or future licenses or agreements or material relationships or any in-licenses upon which our current or future licenses and intellectual property are based are terminated or breached, we may:

•lose our rights to develop and market our current and any future product candidates;

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•lose our rights to patent protection for our current or any future product candidates;

•experience significant delays in the development or commercialization of our current or any future product candidates;

•not be able to obtain any other licenses on acceptable terms, if at all; or

•incur liability for damages.

These risks apply to any agreements that we may enter into in the future for MLE-301 or for any future product candidates. If we experience any of the foregoing, it would have a material adverse effect on our business, financial condition and results of operations.

If we fail to comply with our obligations in the agreements under which we hold or license intellectual property rights from third-parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license and intellectual property rights that are important to our business.

Further, we cannot provide any assurances that third-party patents or other intellectual property rights do not exist, which might be enforced against our current product candidates, 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. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, prospects, financial condition and results of operations.

***If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.***

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates. 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 current and future product candidates in the United States and other countries in which we plan to develop and commercialize such product candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our development programs and product candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

Pursuant to the UM License Agreement, we obtained an exclusive, worldwide license to develop, manufacture and commercialize nevanimibe. However, the UM License Agreement permits the University of Michigan, and other non-profit research institutions which are granted such rights from the University of Michigan, to manufacture and research nevanimibe for internal research, public service and internal educational purposes, all of which could result in new patentable inventions concerning the manufacture or use of nevanimibe. In addition, pursuant to the Roche License Agreement, we obtained an exclusive, worldwide license to develop, manufacture and commercialize MLE-301. However, the Roche License Agreement permits Roche to use MLE-301 for internal research purposes, which could result in new patentable inventions concerning the manufacture or use of MLE-301.

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. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our current and future product candidates in the United States or in other foreign countries. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue and even if such patents cover our current and future product candidates, third-parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed, invalidated or held unenforceable. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate and companion diagnostic under patent protection could be reduced.

If the patent applications we hold or have in-licensed with respect to our development programs and product candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our current and future product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize future drugs. Any such outcome could have a material adverse effect on our business.

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The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States, or vice versa. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Further, we may not be aware of all third-party intellectual property rights potentially relating to our product candidates. Publications of discoveries in scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically published 18 months after filing, or in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates, in whole or in part, or which effectively prevent others from commercializing competitive technologies and drugs. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act (the “Leahy-Smith Act”), was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The United States Patent and Trademark Office (“USPTO”), recently 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 to file provisions, only 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 patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition. Any further changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents and patent applications or narrow the scope of our potential patent protection.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third-parties to commercialize our technology or product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize product candidates without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed 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 freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and product candidates, or limit the duration of the patent protection of our technology and product candidates. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years from the earliest filing date of a non-provisional patent application. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such drugs. 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, we owned and licensed patent portfolio may not provide it with sufficient rights to exclude others from commercializing drugs similar or identical to that of us.

***We jointly own patents and patent applications with third-parties. Our ability to exploit or enforce these patent rights, or to prevent the third-party from granting licenses to others with respect to these patent rights, may be limited in some circumstances.***

We jointly own certain patents and patent applications with third-parties. In the absence of an agreement with each co-owner of jointly owned patent rights, we will be subject to default rules pertaining to joint ownership. Some countries require the consent of all joint owners to exploit, license or assign jointly owned patents, and if we are unable to obtain that consent from the joint owners, we may be unable to exploit the invention or to license or assign our rights under these patents and patent applications in those countries. For example, we secured exclusive rights from the University of Michigan for certain patents and patent applications that they jointly own with us related to nevanimibe. Additionally, in the United States, each co-owner may be

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required to be joined as a party to any claim or action we may wish to bring to enforce these patent rights, which may limit our ability to pursue third-party infringement claims.

***We have in-licensed patents and patent applications from third-parties. Our ability to exploit or enforce these patent rights, or to prevent the third-party from granting licenses to others with respect to these patent rights, may be limited in some circumstances.***

We have in-licensed certain patents and patent applications from third-parties. In the absence of an agreement with each patent rights owner, we will be subject to default rules pertaining to ownership. Some countries require the consent of all owners to exploit, license or assign owned patents, and if we are unable to obtain that consent from the owners, we may be unable to exploit the invention or to license or assign our rights under these patents and patent applications in those countries. For example, we secured exclusive rights from Roche for certain patents and patent applications that they own related to MLE-301. Additionally, in the United States, each owner may be required to be joined as a party to any claim or action we may wish to bring to enforce these patent rights, which may limit our ability to pursue third-party infringement claims.

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

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States in several stages over the lifetime of our owned and licensed patents and/or applications and any patent rights it may own or license in the future. We rely on our outside counsel or our licensing partners to pay these fees due 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 would have a material adverse effect on our business.

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

Given the amount of time required for the development, testing and regulatory review of new product candidates such as MLE-301, patents protecting such candidates might expire before or shortly after such candidates are commercialized. We have in the past sought, and in the future expect to seek, extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits extension of the term of one U.S. patent that includes at least one claim covering the composition of matter of an FDA-approved drug, an FDA-approved method of treatment using the drug. The extended patent term cannot exceed the shorter of five years beyond the non-extended expiration of the patent or 14 years from the date of the FDA approval of the drug. However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. Further, we may not elect to extend the most beneficial patent to us or the claims underlying the patent that we choose to extend could be invalidated. If any of the foregoing occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing its clinical and preclinical data and launch their drug earlier than might otherwise be the case.

***Intellectual property rights do not necessarily address all potential threats to our business.***

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. The following examples are illustrative:

•others may be able to make compounds or formulations that are similar to our MLE-301 formulation but that are not covered by the claims of the patents that we own or control;

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•we or any strategic partners might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or control;

•we might not have been the first to file patent applications covering certain of our inventions;

•others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;

•it is possible that our pending patent applications will not lead to issued patents;

•issued patents that we own or control may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;

•our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights and then use the information learned from such activities to develop competitive drugs for sale in our major commercial markets;

•we may not develop additional proprietary technologies that are patentable; and

•the patents of others may have an adverse effect on our business.

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

Our commercial success depends, in part, upon our ability, and the ability of our future collaborators, to develop, manufacture, market and sell MLE-301 and any future product candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of 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 MLE-301 and any future product candidates and technology, including interference proceedings, post grant review and inter partes review before the USPTO. 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 have a material adverse effect on our ability to commercialize MLE-301 and any future product candidates. 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 candidate 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 candidate. 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 MLE-301 or any future product candidates or force us to cease some or all of our business operations, which would have a material adverse effect on our business. Claims that we have misappropriated the confidential information or trade secrets of third-parties could have a similar material adverse effect on our business. Even if we prevail in such infringement claims, patent litigation can be expensive and time consuming, which would harm our business, financial condition and results of operations.

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

Competitors may infringe or otherwise violate our patents, the patents of our licensors or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is

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unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of ours patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. The initiation of a claim against a third-party may also cause the third-party to bring counter claims against us such as claims asserting that our patents are 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, non-enablement or lack of statutory subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third-parties may also raise similar validity claims before the USPTO in post-grant proceedings such as ex parte reexaminations, inter partes review, or post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we have licensed, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third-party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our current or future product candidates. Such a loss of patent protection could have material adverse effect on our business.

We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Our business could be harmed if in litigation the prevailing party does not offer us a license on commercially reasonable terms. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees. Even if we prevail in such infringement claims, patent litigation can be expensive and time consuming, which would harm our business, financial condition and results of operations.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have an adverse effect on the price of our common stock.

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

The United States has recently enacted and implemented wide-ranging patent reform legislation. The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, federal courts, USPTO, and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we have licensed or that we might obtain in the future. Similarly, changes in patent law and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we have licensed or that we may obtain in the future.

***We may not be able to protect our intellectual property rights throughout the world, which could have a material adverse effect on our business.***

Filing, prosecuting and defending patents covering MLE-301 and any future product candidates throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop our own drugs and, further, may export otherwise infringing drugs to territories where we may obtain patent protection, but where patent enforcement is not as strong as that in the United States. These drugs may compete with our drugs in jurisdictions where we do not have any issued or licensed patents and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

***Our reliance on third-parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.***

If we rely on third-parties to manufacture and commercialize MLE-301 or any future product candidates, or if we collaborate with third-parties for the development of MLE-301 or any future product candidates, we must, at times, share trade secrets with

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them. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third-parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third-parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor’s discovery of our trade secrets or other unauthorized use or disclosure could have an adverse effect on our business and results of operations.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third-parties, independent development or publication of information by any of third-party collaborators. A competitor’s discovery of our trade secrets would harm our business.

***We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of their former employers or other third-parties.***

Certain 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. 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 approach 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.

**Risks Related to Our Dependence on Third-Parties**

***We do not have our own manufacturing capabilities and will rely on third-parties to produce clinical and commercial supplies of MLE-301 and any future product candidates.***

We have no experience in drug formulation or manufacturing and do not own or operate, and we do not expect to own or operate, facilities for product manufacturing, storage and distribution, or testing. We currently rely on contract manufacturing organizations (“CMOs”), to produce MLE-301 for our clinical trials, including the recently initiated Phase 1 clinical trial of MLE-301. Additionally, we rely on CMOs with respect to the manufacture of drug product for our clinical trials, including for filing and packaging. Any significant delay in the supply of a product candidate, or the raw material components thereof, for an ongoing clinical trial due to the need to replenish the supply or replace a third-party manufacturer could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our product candidates. If we or our manufacturer are unable to purchase these raw materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenue from the sale of our product candidates.

We will need to rely on third-party manufacturers to supply us with sufficient quantities of MLE-301 to be used, if approved, for its commercialization. The facilities used by our contract manufacturers to manufacture our product candidates must be approved by the FDA pursuant to inspections that will be conducted after we submit our NDA to the FDA. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with cGMP requirements for manufacture of drug products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to secure or maintain regulatory approval for their manufacturing facilities. In addition, we have no control over the ability of our contract

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manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved. Further, our reliance on third-party manufacturers entails risks, to which we would not be subject if we manufactured product candidates ourselves, including:

•inability to meet our product specifications and quality requirements consistently;

•delay or inability to procure or expand sufficient manufacturing capacity;

•issues related to scale-up of manufacturing;

•costs and validation of new equipment and facilities required for scale-up;

•failure to comply with cGMP and similar foreign standards;

•inability to negotiate manufacturing agreements with third-parties under commercially reasonable terms;

•termination or nonrenewal of manufacturing agreements with third-parties in a manner or at a time that is costly or damaging to us;

•reliance on a limited number of sources, and in some cases, single sources for product components;

•lack of qualified backup suppliers for those materials that are currently purchased from a sole or single source supplier;

•operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including public health emergencies, such as the COVID-19 pandemic, natural disasters, such as earthquakes, fires or floods, or the bankruptcy of the manufacturer or supplier;

•inability to find replacement manufacturers or suppliers, if necessary, on terms favorable to us, in a timely manner, or at all;

•carrier disruptions or increased costs that are beyond our control; and

•failure to deliver our products under specified storage conditions and in a timely manner.

Any of these events could lead to clinical trial delays, failure to obtain regulatory approval or impact our ability to successfully commercialize our products once approved. Some of these events could be the basis for FDA or other regulatory authority action, including injunction, recall, seizure, or total or partial suspension of production.

***We may in the future enter into collaborations with third-parties to develop our product candidates. If these collaborations are not successful, our business could be harmed.***

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

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

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to attract new collaborators and the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization apply to the activities of our potential future collaborators.

***We may not be successful in finding strategic collaborators for continuing development of MLE-301 or successfully commercializing or competing in the market for certain diseases.***

We may seek to develop strategic partnerships for developing and commercializing MLE-301, due to capital costs required to develop the product candidate, manufacturing constraints or anticipated commercialization costs. We may not be successful in our efforts to establish such a strategic partnership or other alternative arrangements for MLE-301 because our research and development pipeline may be insufficient or third-parties may not view MLE-301 as having the requisite potential to demonstrate safety and efficacy. In addition, we may be restricted under an existing collaboration agreement from entering into a future agreement with a potential collaborator. We cannot be certain that, following a strategic transaction or license, we will achieve an economic benefit that justifies such transaction.

If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms or at all, we may have to curtail the development of our product candidates, reduce or delay the development programs, delay potential commercialization, 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 expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop MLE-301, which could harm our business, financial condition and results of operations.

***We rely on third-parties to conduct, supervise and monitor our clinical trials, and if those third-parties perform in an unsatisfactory manner, it may harm our business.***

We currently do not have the ability to independently conduct preclinical studies and clinical trials that comply with the regulatory requirements known as good laboratory practice (“GLP”), and good clinical practice (“GCP”), respectively. We also do not currently have the ability to independently conduct large clinical trials. We rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, including our recently initiated Phase 1 clinical trial of MLE-301, and we expect to have limited influence over their actual performance.

We rely upon CROs to monitor and manage data for our clinical programs, as well as the execution of future preclinical studies. We expect to control only certain aspects of our CROs’ activities. Nevertheless, we are responsible for ensuring that each of our studies or trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards and our reliance on the CROs does not relieve us of our regulatory responsibilities.

We and our CROs are required to comply with GLP and GCP, which are regulations and guidelines enforced by the FDA and are also required by the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities in the form of International Conference on Harmonization guidelines for any of our product candidates that are in preclinical and clinical development, respectively. The regulatory authorities enforce GCP through periodic inspections of trial sponsors, principal investigators and clinical trial sites. Although we rely on CROs to conduct GLP-compliant preclinical and preclinical studies and current or planned GCP-compliant clinical trials, we remain responsible for ensuring that each of our GLP preclinical studies and clinical trials is conducted in accordance with our investigational plan and protocol and applicable laws and regulations, and our reliance on the CROs does not relieve us of our regulatory responsibilities. If we or our CROs fail to comply with GCP, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of subjects, we may be required to repeat clinical trials, which would delay the regulatory approval process.

While we have agreements governing their activities, our CROs are not our employees, and we do not control whether or not they devote sufficient time and resources to our clinical and preclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials, or other drug development activities which could harm our business. We face the risk of potential unauthorized disclosure or misappropriation of our intellectual property by CROs, which may reduce our trade secret protection and allow our potential competitors to access and exploit our proprietary technology. In addition, our CROs may experience business disruptions from public health emergencies, such as the COVID-19 pandemic and accompany shelter-in-place or similar orders. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of

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the clinical data they obtain is compromised due to the failure to adhere to its clinical protocols or regulatory requirements or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize any product candidate that we develop. As a result, our financial results and the commercial prospects for any product candidate that we develop would be harmed, our costs could increase, and our ability to generate revenue could be delayed.

If our relationships with these CROs terminate, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can negatively impact our ability to meet our desired clinical development timelines. In addition, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms as a result of business disruptions from public health emergencies, such as the COVID-19 pandemic. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a negative impact on our business and financial condition. Further, we currently rely on several CROs to conduct our ongoing clinical trials and may engage one of these same CROs to conduct additional clinical trials on our behalf. To the extent that these CROs fail to comply with GLP or their contractual obligations to us for any reason, the negative impact on our business and financial condition could be more profound than if we relied on a greater number of CROs.

**Risks Related to Our Business Operations, Employee Matters and Managing Growth**

***Our business, preclinical studies and clinical development programs and timelines, our financial condition and results of operations could be materially and adversely affected by the current COVID-19 pandemic.***

A novel strain of coronavirus, SARS-CoV-2, causing COVID-19, has been declared a pandemic by the World Health Organization. The COVID-19 pandemic has resulted in travel and other restrictions in order to reduce the spread of the disease, including state and local orders across the country, which, among other things, direct individuals to shelter at their places of residence, direct businesses and governmental agencies to cease non-essential operations at physical locations, prohibit certain non-essential gatherings, and order cessation of non-essential travel. In response to these public health directives and orders, we have implemented work-from-home policies for certain employees. The effects of the executive orders, the shelter-in-place orders and our work-from-home policies may negatively impact productivity, disrupt our business and delay our clinical programs and timelines, the magnitude of which will depend, in part, on the length and severity of the restrictions and other limitations on our ability to conduct our business in the ordinary course. These and similar, and perhaps more severe, disruptions in our operations could negatively impact our business, operating results and financial condition.

Quarantines, shelter-in-place and similar government orders related to COVID-19 may adversely impact our business operations and the business operations of our contract research organizations conducting our clinical trials and our third-party manufacturing facilities in the United States and other countries. In particular, some of our third-party manufacturers which we use for the supply of materials for product candidates or other materials necessary to manufacture product to conduct preclinical studies and clinical trials are located in countries affected by COVID-19, and should they experience disruptions, such as temporary closures or suspension of services, we would likely experience delays in advancing these tests and trials. Currently, we expect no material impact on the clinical supply of MLE-301.

In addition, our clinical trials may be affected by the COVID-19 pandemic. For example, clinical site initiation and patient enrollment may be delayed due to prioritization of hospital resources toward the COVID-19 pandemic. Some patients may not be able to comply with clinical trial protocols if quarantines impede patient movement or interrupt healthcare services. Similarly, our ability to recruit and retain patients and principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19 and adversely impact our clinical trial operations. As a result, we may face delays in meeting our anticipated timelines for our ongoing and planned clinical trials.

The spread of COVID-19, which has caused a broad impact globally, may materially affect us economically. While the potential economic impact brought by, and the duration of, COVID-19 may be difficult to assess or predict, a widespread pandemic could result in significant disruption of global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity. In addition, a recession or market correction resulting from the spread of COVID-19 could materially affect our business and the value of our common stock.

The global pandemic of COVID-19 continues to rapidly evolve. The extent to which the COVID-19 pandemic impacts our business, our clinical development and regulatory efforts will depend on future developments that are highly uncertain and cannot be predicted with confidence, such as the duration of the outbreak, travel restrictions, quarantines, social distancing requirements and business closures in the United States and other countries, and business disruptions, and the effectiveness of

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actions taken in the United States and other countries to contain and treat the disease. Accordingly, we do not yet know the full extent of potential delays or impacts on our business, our clinical and regulatory activities, healthcare systems or the global economy as a whole. However, these impacts could adversely affect our business, financial condition, results of operations and growth prospects.

In addition, to the extent the ongoing COVID-19 pandemic adversely affects our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described in this ‘‘Risk Factors’’ section.

***Potential future acquisitions could prove difficult to integrate, disrupt our business, dilute stockholder value and strain our resources.***

We are seeking opportunities for a potential expansion of our pipeline, and as a result of such activities, we may acquire companies, technologies or product candidates that we believe could complement or expand our business. Integrating the operations of acquired businesses successfully or otherwise realizing any of the anticipated benefits of acquisitions involves a number of potential challenges. The failure to meet these integration challenges could seriously harm our financial condition and results of operations. Realizing the benefits of acquisitions depends in part on the integration of operations and personnel. These integration activities are complex and time-consuming, and we may encounter unexpected difficulties or incur unexpected costs, including with respect to:

•diversion of management attention from ongoing business concerns to integration matters;

•coordinating clinical and preclinical development plans;

•consolidating and rationalizing information technology and accounting platforms and administrative infrastructures;

•complexities associated with managing the geographic separation of the combined businesses and consolidating multiple physical locations;

•reconciling different corporate cultures; and

•retaining scientific and other key employees.

Acquired businesses may have liabilities, adverse operating issues or other matters of concern arise following the acquisition that we fail to discover through due diligence prior to the acquisition. Further, our acquisition targets may not have as robust internal controls over financial reporting as would be expected of a public company. Acquisitions may also result in the recording of goodwill and other intangible assets that are subject to potential impairment in the future that could harm our financial results. We may also become subject to new regulations as a result of an acquisition, including if we acquire operations in a country in which we do not already operate. If we fail to properly evaluate acquisitions or unanticipated issues arise following the acquisition, we may incur costs in excess of what we anticipate and may not otherwise achieve the anticipated benefits of any such acquisitions.

***We are highly dependent*** ***on the services of our key executives and personnel, including Julia C. Owens, Ph.D., our chief executive officer, Christophe Arbet-Engels, M.D., Ph.D., our chief medical officer, and Ryan Zeidan, Ph.D., our chief development officer, and if we are not able to retain these members of our management team or recruit and retain additional management, clinical and scientific personnel, our business will be harmed.***

We are highly dependent on Drs. Owens, Arbet-Engels, and Zeidan. The employment agreements we have with these officers do not prevent such persons from terminating their employment with us at any time. Further, these officers may be unable to perform their duties or have limited availability due to COVID-19 or other health emergencies. The temporary or permanent loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

In addition, we are dependent on our continued ability to attract, retain and motivate highly qualified additional management, clinical and scientific personnel. If we are not able to retain our management and to attract, on acceptable terms, additional qualified personnel necessary for the continued development of our business, we may not be able to sustain our operations or grow.

We may not be able to attract or retain qualified personnel in the future due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses. Many of the other pharmaceutical companies that we compete

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against for qualified personnel and consultants have greater financial and other resources, different risk profiles, are located in geographies with a larger biotechnology industry presence and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates and consultants than what we have to offer. If we are unable to continue to attract, retain and motivate high-quality personnel and consultants to accomplish our business objectives, the rate and success at which we can discover and develop product candidates and our business will be limited and we may experience constraints on our development objectives.

Our future performance will also depend, in part, on our ability to successfully integrate newly hired executive officers into our management team and our ability to develop an effective working relationship among senior management. Our failure to integrate these individuals and create effective working relationships among them and other members of management could result in inefficiencies in the development and commercialization of our product candidates, harming future regulatory approvals, sales of our product candidates and our results of operations. Additionally, we do not currently maintain “key person” life insurance on the lives of our executives or any of our employees.

***We must attract and retain highly skilled employees to succeed.***

To succeed, we must recruit, develop, retain, manage and motivate qualified clinical, scientific, technical, general and administrative and management personnel while facing significant competition for experienced personnel. In April 2020, we announced our decision to discontinue the development of livoletide as a potential treatment for PWS. In connection with the discontinuation of the livoletide program in PWS, we eliminated employee positions representing approximately 30% of our prior headcount (the “restructuring”). The restructuring could harm our ability to attract and retain qualified personnel. The restructuring could also result in reduced morale and productivity among our remaining personnel. Our inability or failure to successfully attract and retain qualified personnel, particularly at the management level, could adversely affect our ability to execute our business plan and harm our operating results. In particular, the loss of one or more of our executive officers could be detrimental if we cannot recruit suitable replacements in a timely manner. The competition for qualified personnel in the pharmaceutical field is intense and we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel.

Many of the other pharmaceutical companies with which we compete for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we have. These companies may also provide more diverse opportunities and better or more chances for development or career advancement. Some of these characteristics may appeal more to high-quality candidates than what we offer. If we are unable to continue to attract and retain personnel, the rate at which we can discover, develop and advance current and future product candidates, and our success in doing so, will be limited and our business may be harmed.

***Our employees, independent contractors, principal investigators, consultants, commercial collaborators, service providers and other vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have an adverse effect on our results of operations.***

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 intentional failures to comply with FDA regulations or the regulations applicable in other jurisdictions, provide accurate information to the FDA and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter employee 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 itself or asserting our rights, those actions could have a negative impact on our business, financial condition and results of operations, including the imposition of significant fines or other sanctions.

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***We may be delayed in our receipt of certain tax benefits that Alizé historically received as a French technology company.***

As a French technology company, Alizé historically benefited from certain tax advantages, including the French research tax credit (*credit d’impot recherche*) (“CIR”). The CIR is a French tax credit aimed at stimulating research and development and can offset French corporate income tax due. Alizé has historically received CIR reimbursements promptly following filing for such reimbursements with applicable French taxing authorities. For the year ended December 31, 2018, claims were made totaling $1.3 million, which we received in the third quarter of 2019. We filed claims totaling $1.3 million for the year ended December 31, 2019, which we received in the second quarter of 2020. In the future, we may no longer qualify as a French small or medium size enterprise, and, accordingly, we may be subject to a three-year waiting period for reimbursement of CIRs, which could adversely affect the combined business’s results of operations and cash flows. In addition, the amount of CIR received is, among other factors, dependent upon incurring qualified research and development expenses and maintaining a certain level of employee salaries and other personnel costs in France. The number of our research and development employees in France decreased during the year ended December 31, 2019 and we will experience a decrease in qualified research and development expenses for the year ending December 31, 2020 due to the discontinuation of our livoletide program. Therefore, the amount of CIR we are eligible for will decrease.

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

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we are not aware of 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.

***We may be exposed to significant foreign exchange risk.***

We incur portions of our expenses, and may in the future derive revenue, in currencies other than the U.S. dollar, in particular, the euro. As a result, we are exposed to foreign currency exchange risk as our results of operations and cash flows are subject to fluctuations in foreign currency exchange rates. Any fluctuation in the exchange rate of these foreign currencies may negatively impact our business, financial condition and operating results. Global economic events, such as the COVID-19 pandemic, have and may continue to significantly impact local economies and the foreign exchange markets, which may increase the risks associated with sales denominated in foreign currencies. We currently do not engage in hedging transactions to protect against uncertainty in future exchange rates between particular foreign currencies and the euro. Therefore, for example, an increase in the value of the euro against the U.S. dollar could be expected to have a negative impact on our operating expenses as euro denominated expenses, if any, would be translated into U.S. dollars at an increased value. We cannot predict the impact of foreign currency fluctuations, and foreign currency fluctuations in the future may adversely affect our financial condition, results of operations and cash flows.

***The risks arising with respect to the historic OvaScience business and operations may be different from what we anticipate, which could lead to significant, unexpected costs and liabilities and could materially and adversely affect our business going forward.***

It is possible that we may not have fully anticipated the extent of the risks associated with the Merger we completed with OvaScience in 2018. After the Merger, OvaScience’s historic business was discontinued, but prior to the transaction OvaScience had a significant operating history. As a consequence, we may be subject to claims, demands for payment, regulatory issues, costs and liabilities that were not and are not currently expected or anticipated. Notwithstanding our exercise of due diligence pre-transaction and winding down of the OvaScience business post-transaction, the risks involved with taking over a business with a significant operating history and the costs and liabilities associated with these risks may be greater than we anticipate. We may not be able to contain or control the costs or liabilities associated with OvaScience’s historic business, which could materially and adversely affect our business, liquidity, capital resources or results of operation.

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**Risks Related to Ownership of Our Common Stock and Our Status as a Public Company**

***The trading price of the shares of our common stock has been and is likely to continue to be volatile, and purchasers of our common stock could incur substantial losses.***

The market price of our common stock has been and is likely to continue to be highly volatile and could be subject to wide fluctuations in price in response to various factors. A number of factors could influence the volatility in the trading price of our common stock, including changes in the economy or in the financial markets, including recently in connection with the ongoing COVID-19 pandemic, industry-related developments, and the impact of material events and changes in our operations, including as a result of our recent announcements that we have discontinued our livoletide program in PWS and ceased investing in our nevanimibe program. Worsening economic conditions and other adverse effects or developments relating to our business or the ongoing COVID-19 pandemic may negatively affect the market price of our common stock. The market price for our common stock is likely to continue to be volatile, particularly due to the ongoing COVID-19 pandemic, and subject to significant price and volume fluctuations in response to market, industry and other factors, including the risk factors described in this “Risk Factors” section. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

•the commencement, enrollment or results of our clinical trials or changes in the development status of our product candidates;

•any delay in our regulatory filings for any product candidate we may develop, and any adverse development or perceived adverse development with respect to the applicable regulatory authority’s review of such filings, including without limitation the FDA’s issuance of a “refusal to file” letter or a request for additional information;

•adverse results from, delays in or termination of clinical trials;

•adverse regulatory decisions, including failure to receive regulatory approval of our product candidates;

•unanticipated serious safety concerns related to the use of our product candidates;

•changes in financial estimates by us or by any securities analysts who might cover our stock;

•conditions or trends in our industry;

•changes in the structure of healthcare payment systems;

•changes in the market valuations of similar companies;

•stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;

•publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;

•announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;

•announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;

•investors’ general perception of our company and our business;

•recruitment or departure of key personnel;

•overall performance of the equity markets;

•trading volume of our common stock;

•disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;

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•significant lawsuits, including patent or stockholder litigation;

•general political and economic conditions; and

•other events or factors, many of which are beyond our control.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies’ stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management’s attention and resources from our business.

***If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.***

The trading market for our common stock will be influenced by the research and reports that equity research analysts publish about us and our business. As a newly public company, we have only limited research coverage by equity research analysts. Equity research analysts may elect not to initiate or continue to provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. Even if we continue to have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

***Future sales of our common stock in the public market could cause our share price to decline.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time, subject to the restrictions and limitations described below. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that sales, particularly sales by our directors, executive officers, and significant stockholders, may have on the prevailing market price of our common stock. As of September 30, 2020, we had 18,999,701 shares of common stock outstanding. All of our outstanding shares of common stock are available for sale in the public market, subject only to the restrictions of Rule 144 under the Securities Act. In addition, the shares of common stock subject to outstanding options under our equity incentive plans and the shares reserved for future issuance under our equity incentive plans will become eligible for sale in the public market in the future, subject to certain legal and contractual limitations. In addition, certain holders of our common stock have the right, subject to various conditions and limitations, to request we include their shares of our common stock in registration statements we may file relating to our securities.

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

•establish a classified board of directors such that 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 for nominations to our board of directors;

•limit who may call stockholder meetings;

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•prohibit actions by our stockholders by written consent;

•require that stockholder actions be effected at a duly called stockholders meeting;

•authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and

•require the approval of the holders of at least 75 percent of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our certificate of incorporation or by-laws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns 15 percent or more of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired 15 percent or more of our outstanding voting stock, unless the merger or combination is approved in a manner prescribed by the statute.

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

As of September 30, 2020, our executive officers, directors and current beneficial owners of 5% or more of our common stock and their respective affiliates, in the aggregate, beneficially own approximately 33.0% of our outstanding common stock. As a result, these persons, acting together, can significantly influence all matters requiring stockholder approval, including the election and removal of directors, any merger, consolidation, sale of all or substantially all of our assets, or other significant corporate transactions.

Some of these persons or entities may have interests different than yours. For example, because many of these stockholders purchased their shares at prices substantially below the current market price of our common stock and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors, or they may want us to pursue strategies that deviate from the interests of other stockholders.

***We are at risk of securities class action and similar litigation.***

In the past, securities class action litigation has often been brought against a company following a decline in the market price of our securities. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. We remain the subject of various securities class action lawsuits and shareholder derivative lawsuits that were filed against OvaScience and certain of its officer and directors, as described in more detail in Item 3, Legal Proceedings. These lawsuits, as well as any similar lawsuits initiated in the future, could result in substantial cost and a diversion of management’s attention and resources, which could harm our business.

***If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.***

We are subject to the reporting requirements of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, and the rules and regulations of the stock market on which our common stock is listed. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting and that we furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting. This assessment includes disclosure of any material weaknesses identified by our management in our internal control over financial reporting. However, due to recent changes in SEC rules related to smaller reporting companies, we do not expect to be required to have our auditors formally attest to the effectiveness of our internal control over financial reporting in connection with our Annual Report on Form 10-K for the year ending December 31, 2020. For the year ended December 31, 2018, we were unable to conduct the required assessment primarily due to the Merger occurring in the fourth quarter of 2018 and the substantial change in operational focus, management and the internal control environment following the Merger. As a result, we provided our first internal control assessment with our Annual Report on Form 10-K for the year ended December 31, 2019.

We may identify weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system’s objectives will be met. Because of the inherent limitations in all control systems, no

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evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the Securities and Exchange Commission (“SEC”), or other regulatory authorities.

***We expect to continue to incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to compliance with our public company responsibilities and corporate governance practices.***

As a relatively new public company, we continue to incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Capital Market and other applicable securities rules and regulations impose various requirements on public companies. Our management and other personnel need to devote a substantial amount of time to compliance with these requirements. Moreover, these rules and regulations 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 directors’ and officers’ liability insurance, compared to when we were a private company, which could make it more difficult for us to attract and retain qualified members of our board of directors. We cannot predict or estimate the amount of additional costs we will continue to incur as a public company or the timing of such costs.

***Changes in tax laws or regulations could materially adversely affect our company.***

New tax laws or regulations could be enacted at any time, and existing tax laws or regulations could be interpreted, modified or applied in a manner that is adverse to us, which could adversely affect our business and financial condition. For example, legislation enacted in 2017, informally titled the Tax Act, enacted many significant changes to the U.S. tax laws, including changes in corporate tax rates, the utilization of our NOLs and other deferred tax assets, the deductibility of expenses, and the taxation of foreign earnings. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. For example, the CARES Act modified certain provisions of the Tax Act. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the CARES Act, or any newly enacted federal tax legislation. The impact of changes under the Tax Act, the CARES Act, or future reform legislation could increase our future U.S. tax expense and could have a material adverse impact on our business and financial condition.

***Our effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.***

We are subject to taxation in more than one tax jurisdiction. As a result, our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate. In preparing our financial statements, we estimate the amount of tax that will become payable in each of such places. Nevertheless, our effective tax rate may be different than experienced in the past due to numerous factors, including passage of the newly enacted federal income tax law, changes in the mix of our profitability from jurisdiction to jurisdiction, the results of examinations and audits of our tax filings, our inability to secure or sustain acceptable agreements with tax authorities, changes in accounting for income taxes, cash repatriation restrictions and possible withholding taxes and changes in tax laws. Any of these factors could cause us to experience an effective tax rate significantly different from previous periods or our current expectations and may result in tax obligations in excess of amounts accrued in our financial statements.

***Our ability to use net operating losses and certain other tax attributes to offset future taxable income may be subject to limitation.***

As of December 31, 2019, we had federal and state net operating loss carryforwards (“NOLs”) of $298.6 million and $262.5 million, respectively. Our NOLs could expire unused and be unavailable to offset future income tax liabilities because of their limited duration or because of restrictions under U.S. tax law. Our NOLs generated in tax years ending on or prior to December 31, 2017 are permitted to be carried forward for only 20 years under applicable U.S. tax law. Our federal NOLs generated in tax years ending after December 31, 2017 may be carried forward indefinitely, but the deductibility of federal NOLs generated in tax years beginning after December 31, 2020 is subject to certain limitations. It is uncertain if and to what extent various states will conform to the Tax Act. Our federal and state net operating loss carryforwards will begin to expire, if not utilized, by 2031.

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In addition, under Section 382 and Section 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an “ownership change,” its ability to use its pre-change NOL carryforwards and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. A Section 382 “ownership change” generally occurs if one or more stockholders or groups of stockholders who own at least 5% of our stock increase their ownership by more than 50 percentage points (by value) over their lowest ownership percentage over a rolling three-year period. We may have experienced ownership changes in the past and may experience ownership changes in the future as a result of shifts in our stock ownership (some of which are outside our control). As a result, if we earn net taxable income, our ability to use our pre-change NOLs to offset such taxable income may be subject to limitations. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Consequently, even if we achieve profitability, we may not be able to utilize a material portion of our net operating loss carryforwards and certain other tax attributes, which could have a material adverse effect on cash flow and results of operations.

***We do not anticipate paying any cash dividends on our common stock in the foreseeable future.***

You should not rely on an investment in our common stock to provide dividend income. We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. In addition, the terms of any existing or future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our common stock.

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

**Recent Sales of Unregistered Securities**

We did not sell any unregistered securities during the three months ended September 30, 2020.

**Issuer Purchases of Equity Securities**

We did not repurchase any securities during the three months ended September 30, 2020.

**Item 3. Defaults upon Senior Securities**

Not applicable.

**Item 4. Mine Safety Disclosures**

Not applicable.

**Item 5. Other Information**

Not applicable.

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**Item 6. Exhibits**

The following exhibits are incorporated by reference or filed as part of this report.

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |
| **Exhibit**  **Number** | | |  | | | **Description** | | |
| 3.1 | | |  | | | [Restated Certificate of Incorporation of the Registrant, as amended (incorporated by reference from Exhibit 3.1 to the Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 15, 2019 File No. 001-35890)](https://www.sec.gov/Archives/edgar/data/1544227/000155837019005038/mlnd-20190331ex31b53fa83.htm) | | |
|  | | |  | | |  | | |
| 3.2 | | |  | | | [Third Amended and Restated Bylaws, as Amended, of the Registrant (incorporated by reference from Exhibit 3.1 to the Current Report on Form 8-K filed with the Securities and Exchange Commission on August 9, 2018 File No. 001-35890)](https://www.sec.gov/Archives/edgar/data/1544227/000110465918050995/a18-18482_1ex3d1.htm) | | |
|  | | |  | | |  | | |
| 31.1\* | | |  | | | [Certification of Chief Executive Officer (Principal Executive Officer) pursuant to Section 302 of Sarbanes-Oxley Act of 2002](mlnd-20200930x10qexhibit311.htm) | | |
|  | | |  | | |  | | |
| 31.2\* | | |  | | | [Certification of Chief Financial Officer (Principal Financial Officer) pursuant to Section 302 of Sarbanes-Oxley Act of 2002](mlnd-20200930x10qexhibit312.htm) | | |
|  | | |  | | |  | | |
| 32.1+ | | |  | | | [Certification of Chief Executive Officer (Principal Executive Officer) and Chief Financial Officer (Principal Financial Officer) pursuant to Section 906 of Sarbanes-Oxley Act of 2002](mlnd-20200930x10qexhibit321.htm) | | |
|  | | |  | | |  | | |
| 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 - the cover page interactive data is embedded within the Inline XBRL document or included within the Exhibit 101 attachments. | | |

\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

\*    Filed herewith.

+    This certification is being furnished solely to accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, and is not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing of the registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

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**SIGNATURES**

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

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  |  |  |  |
|  | | | MILLENDO THERAPEUTICS, INC. | | | | | |
|  | | |  | | |  | | |
|  | | | By: | | | /s/ Julia C. Owens, Ph.D. | | |
|  | | |  | | | Julia C. Owens, Ph.D. President and Chief Executive Officer (Principal Executive Officer) | | |
|  | | |  | | |  | | |
|  | | | By: | | | /s/ Louis Arcudi III | | |
|  | | |  | | | Louis Arcudi III Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer) | | |
| Date: November 9, 2020 | | |  | | |  | | |

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