

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

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2016
or

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

Commission File Number: 001-37566

Mirna Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction of
incorporation or organization)

26-1824804
(I.R.S. Employer
Identification No.)

3711 South Mopac
Austin, TX (Address of principal executive offices)

78735
(Zip Code)

(512) 901-0900
(Registrant's telephone number, including area code)

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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post such files).
Yes No

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

Large accelerated filer Accelerated filer

Non-accelerated filer (Do not check if a smaller reporting company) Smaller reporting company

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

As of November 7, 2016 there were 20,835,868 shares of the registrant's common stock, par value \$0.001 per share, outstanding.

Mirna Therapeutics, Inc.

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PART I—FINANCIAL INFORMATION**Item 1. Financial Statements****Condensed Financial Statements
Mirna Therapeutics, Inc.
Condensed Balance Sheets****(in thousands, except share and per share data)**

| | September 30, 2016 | December 31, 2015 |
|---|-------------------------------|------------------------------|
| | (Unaudited) | |
| Assets | | |
| Current Assets: | | |
| Cash and cash equivalents | \$ 26,958 | \$ 89,713 |
| Short-term marketable securities | 39,300 | — |
| Prepaid expenses and other current assets | 818 | 829 |
| Total current assets | 67,076 | 90,542 |
| Property and equipment, net | 1,834 | 375 |
| Long-term marketable securities | 400 | — |
| Restricted cash | 2,431 | — |
| Total assets | <u>\$ 71,741</u> | <u>\$ 90,917</u> |
| Liabilities and Stockholders' Equity (Deficit) | | |
| Current Liabilities: | | |
| Accounts payable | \$ 1,153 | \$ 3,687 |
| Accrued expenses | 1,943 | 2,214 |
| Total liabilities | <u>3,096</u> | <u>5,901</u> |
| Stockholders' Equity (Deficit): | | |
| Preferred stock, \$0.001 par value, 5,000,000 shares authorized at September 30, 2016 and December 31, 2015; 0 shares outstanding at September 30, 2016 and December 31, 2015 | — | — |
| Common stock, \$0.001 par value; 250,000,000 shares authorized at September 30, 2016 and December 31, 2015; 20,835,868 and 20,830,555 shares issued and outstanding at September 30, 2016 and December 31, 2015, respectively | 21 | 21 |
| Additional paid in capital | 162,729 | 161,518 |
| Accumulated other comprehensive loss | (8) | — |
| Accumulated deficit | (94,097) | (76,523) |
| Total stockholders' equity | <u>68,645</u> | <u>85,016</u> |
| Total liabilities and stockholders' equity | <u>\$ 71,741</u> | <u>\$ 90,917</u> |

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

Mirna Therapeutics, Inc.
Condensed Statements of Operations and Comprehensive Loss (Unaudited)

(in thousands, except share and per share data)

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|-------------------------------------|------------|------------------------------------|-------------|
| | 2016 | 2015 | 2016 | 2015 |
| Operating expenses: | | | | |
| Research and development | \$ 3,384 | \$ 4,683 | \$ 11,589 | \$ 12,584 |
| General and administrative | 1,940 | 1,556 | 6,119 | 3,618 |
| Total operating expenses | 5,324 | 6,239 | 17,708 | 16,202 |
| Other income (expense): | | | | |
| Interest income | 97 | 8 | 272 | 8 |
| Loss on disposal of assets | (138) | — | (138) | — |
| Total other income (expense) | (41) | 8 | 134 | 8 |
| Net loss | \$ (5,365) | \$ (6,231) | \$ (17,574) | \$ (16,194) |
| Less: Accretion and dividends on convertible preferred stock | — | (1,554) | — | (4,217) |
| Net loss attributable to common stockholders | \$ (5,365) | \$ (7,785) | \$ (17,574) | \$ (20,411) |
| Other comprehensive loss: | | | | |
| Unrealized loss on available for sale securities, net of tax | (14) | — | (8) | — |
| Total other comprehensive loss | (5,379) | (7,785) | (17,582) | (20,411) |
| Net loss per share attributable to common stockholders—basic and diluted | \$ (0.26) | \$ (82.16) | \$ (0.84) | \$ (222.70) |
| Common shares used to compute basic and diluted net loss per share attributable to common stockholders | 20,835,868 | 94,753 | 20,832,727 | 91,650 |

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

Mirna Therapeutics, Inc.
Condensed Statements of Cash Flows (Unaudited)

(in thousands)

| | Nine Months Ended September 30, | |
|--|------------------------------------|-------------|
| | 2016 | 2015 |
| Operating activities | | |
| Net loss | \$ (17,574) | \$ (16,194) |
| Adjustment to reconcile net loss to net cash used in operating activities: | | |
| Depreciation and amortization | 105 | 37 |
| Stock-based compensation | 1,202 | 561 |
| Net amortization of premium/ discounts on marketable securities | 178 | — |
| Changes in operating assets and liabilities: | | |
| Grant reimbursement and other receivables | (123) | 61 |
| Prepaid expenses and other assets | 134 | (217) |
| Accounts payable | (2,883) | 1,290 |
| Accrued expenses | (271) | (57) |
| Net cash used in operating activities | (19,232) | (14,519) |
| Investing activities | | |
| Purchases of marketable securities | (79,786) | — |
| Maturities of marketable securities | 39,900 | — |
| Restricted cash | (2,431) | — |
| Purchases of property and equipment | (1,215) | (187) |
| Net cash used in investing activities | (43,532) | (187) |
| Financing activities | | |
| Proceeds from issuance of convertible preferred stock, net of issuance costs | — | 41,023 |
| Proceeds from the exercise of stock options | 9 | 67 |
| Cash provided by financing activities | 9 | 41,090 |
| Net increase (decrease) in cash and cash equivalents | (62,755) | 26,384 |
| Cash and cash equivalents at beginning of period | 89,713 | 9,319 |
| Cash and cash equivalents at end of period | \$ 26,958 | \$ 35,703 |
| Supplemental disclosures for non- cash investing activities: | | |
| Property & equipment purchased in accounts payable | 412 | — |

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

Mirna Therapeutics, Inc.

Notes to Condensed Financial Statements (Unaudited)

1. Nature of Business and Basis of Presentation

Nature of business

Mirna Therapeutics, Inc. (“Mirna” or “the Company”) is a biopharmaceutical company focused on microRNA-based oncology therapeutics. The Company was incorporated in Delaware in December 2007 as a wholly-owned subsidiary of Asuragen, Inc. (“Asuragen”) and was spun out to existing Asuragen stockholders in December 2009. Following the close of the Company’s Phase 1 clinical trial of MRX34 in September 2016, the Company is evaluating its strategic alternatives focusing on enhancing stockholder value, including the possibility of a merger or sale of the Company, and has suspended further research and development activities to reduce operating expenses while it evaluates these opportunities (see Note 13 for additional discussion.) The Company is located in Austin, Texas.

In October 2015, the Company sold 6,250,000 shares of common stock, \$0.001 par value per share, in an underwritten public offering (the “IPO”) and 2,395,010 shares of common stock in a concurrent private placement, with both offerings at a price of \$7.00 per share. The underwriters of the IPO purchased an additional 704,962 shares of common stock pursuant to their option to purchase additional shares. The Company’s aggregate net proceeds from the IPO were \$43.7 million, after deducting the transaction offering costs and the underwriting discounts incurred. The Company also received net proceeds of \$16.7 million after deducting the offering transaction costs from the concurrent private placement.

The Company continues to be subject to a number of risks common to companies in similar stages of development. Principal among these risks are uncertainties of technological innovations, dependence on key individuals, development of the same or similar technological innovations by the Company’s competitors and protection of proprietary technology. The Company believes that its cash, cash equivalents and marketable securities of \$66.7 million at September 30, 2016 will enable the Company to maintain its current and planned operations for at least the next twelve months.

Basis of presentation

The accompanying interim condensed financial statements as of September 30, 2016 and for the three and nine months ended September 30, 2016 and 2015, and the related interim information contained within the notes to the financial statements, are unaudited. The unaudited interim condensed financial statements have been prepared in accordance with generally accepted accounting principles for interim financial information and on the same basis as the audited financial statements. In the opinion of management, the accompanying unaudited interim condensed financial statements contain all adjustments which include only normal recurring adjustments necessary to state fairly the Company’s financial position as of September 30, 2016, and the results of its operations and cash flows for the interim periods ended September 30, 2016 and 2015. Such adjustments are of a normal and recurring nature. The interim financial data as of September 30, 2016 is not necessarily indicative of the results to be expected for the year ending December 31, 2016, or for any future period.

The accompanying condensed financial statements and related financial information should be read in conjunction with the audited financial statements and related notes thereto for the year ended December 31, 2015 included in the Company’s Form 10-K, most recently filed with the Securities and Exchange Commission on March 30, 2016.

2. Summary of Significant Accounting Policies

Use of estimates

The preparation of financial statements in conformity with U.S. generally accepted accounting principles requires the Company’s management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates.

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Research and development costs

Research and development costs are expensed as incurred. Research and development costs include salaries and personnel-related costs, consulting fees, fees paid for contract research services, the costs of laboratory equipment and facilities, development of intellectual property, license fees and other external costs. The Company accounts for government grants as a reduction of research and development expenses. Government grants are recorded at the time the related research and development costs have been incurred by the Company and, accordingly, become eligible for reimbursement. The Company accrues for government grants that have been earned but not yet received.

Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

Clinical Trial and Pre-Clinical Study Accruals

The Company estimates pre-clinical study and clinical trial expenses pursuant to contracts with research institutions and contract research organizations that conduct and manage pre-clinical studies and clinical trials on the Company's behalf. These estimates are based on the level of service performed and the underlying agreement. Further, the Company accrues expenses related to clinical trials based on the level of patient enrollment and other activities according to the related agreements. The Company monitors patient enrollment levels and other activities to the extent reasonably possible and adjusts estimates accordingly. If actual costs incurred or the timing of services vary from our estimate, we adjust the accrual accordingly.

Stock-based compensation

The Company accounts for its stock-based compensation awards in accordance with ASC Topic 718, *Compensation—Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments to employees, including grants of employee stock options, to be recognized in the statements of operations based on their grant date fair values. For stock options granted to employees and to members of the board of directors for their services on the board of directors, the Company estimates the grant date fair value of each option award using the Black-Scholes option-pricing model. The use of the Black-Scholes option-pricing model requires management to make assumptions with respect to the expected term of the option, the expected volatility of the common stock consistent with the expected life of the option, risk-free interest rates and expected dividend yields of the common stock. For awards subject to service-based vesting conditions, the Company recognizes stock-based compensation expense, net of estimated forfeitures, equal to the grant date fair value of stock options on a straight-line basis over the requisite service period.

Fair value measurements

The Company records money market funds at fair value. ASC Topic 820, *Fair Value Measurements and Disclosures*, establishes a fair value hierarchy for those instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). The hierarchy consists of three levels:

- Level 1 – Unadjusted prices in active markets for identical assets or liabilities.
- Level 2 – Quoted prices for similar assets and liabilities in active markets, quoted prices in markets that are not active, or inputs which are observable, either directly or indirectly, for substantially the full term of the asset or liability.
- Level 3 – Unobservable inputs that reflect the Company's own assumptions about the assumptions market participants would use in pricing the asset or liability in which there is little, if any, market activity for the asset or liability at the measurement date.

The carrying amounts reflected in the balance sheets for cash, prepaid expenses and other current assets, accounts payable, and accrued expenses approximate their fair values at September 30, 2016 and December 31, 2015, due to their short-term nature.

The Company evaluates transfers between levels at the end of each reporting period. There were no transfers of assets or liabilities between Level 1, Level 2 or Level 3 during the three or nine months ended September 30, 2016 or 2015.

Restricted Cash

Restricted cash consists of cash amounts held for specific or limited purposes and, therefore, not available for general operating activities. In June 2016, the Company secured a standby letter of credit of \$2.4 million for the benefit of the landlord in the event of default. The restricted cash consists of cash providing security under the terms of the lease described in Note 11.

Marketable Securities

The Company classifies marketable securities with a remaining maturity when purchased of greater than three months as available-for-sale. Marketable securities with a remaining maturity date greater than one year are classified as non-current. Available-for-sale securities are maintained by an investment manager and may consist of U.S. Treasury securities and government agency securities and corporate debt securities. Available-for-sale securities are carried at fair value with the unrealized gains and losses included in other comprehensive loss as a component of stockholders' equity until realized. Any premium or discount arising at purchase is amortized and/or accreted to interest income and/or expense over the life of the instrument. Realized gains and losses are determined using the specific identification method and are included in other income.

If any adjustment to fair value reflects a decline in value of the investment, the Company considers all available evidence to evaluate the extent to which the decline is "other-than-temporary" and, if so, mark the investment to market through a charge to the Company's statement of operations and comprehensive loss.

Comprehensive loss

Comprehensive loss is composed of net loss and other comprehensive income or loss. Other comprehensive loss consists of unrealized gains and losses on marketable securities.

Recently Issued Accounting Pronouncements

In March 2016, the Financial Accounting Standards Board (FASB) issued ASU 2016-09, *Compensation - Stock Compensation (Topic 718): Improvements to Employee Share Based Payment Accounting* ("ASU 2016-09") as part of the FASB simplification initiative. The new standard provides for changes to accounting for stock compensation including 1) excess tax benefits and tax deficiencies related to share-based payment awards will be recognized as income tax expense in the reporting period in which they occur; 2) excess tax benefits will be classified as an operating activity in the statement of cash flow; 3) the option to elect to estimate forfeitures or account for them when they occur; and 4) increase tax withholding requirements threshold to qualify for equity classification. The ASU is effective for public companies for annual periods, and interim periods within those annual periods, beginning after December 15, 2016, and early adoption is permitted. The Company is currently evaluating the impact that ASU 2016-09 will have on the financial statements.

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842)*. The new standard requires the recognition of assets and liabilities arising from lease transactions on the balance sheet and the disclosure of key information about leasing arrangements. Accordingly, a lessee will recognize a lease asset for its right to use the underlying asset and a lease liability for the corresponding lease obligation. Both the asset and liability will initially be measured at the present value of the future minimum lease payments over the lease term. Subsequent measurement, including the presentation of expenses and cash flows, will depend on the classification of the lease as either a finance or an operating lease. Initial costs directly attributable to negotiating and arranging the lease will be included in the asset. For leases with a term of twelve months or less, a lessee can make an accounting policy election by class of underlying asset to not recognize an asset and corresponding liability. Lessees will also be required to provide additional qualitative and quantitative disclosures regarding the amount, timing and uncertainty of cash flows arising from leases. These disclosures are intended to supplement the amounts recorded in the financial statements and provide additional information about the nature of an organization's leasing activities. The new standard is effective for fiscal years beginning after December 15, 2018, and interim periods within those years, with early adoption permitted. In transition, lessees are required to recognize and measure leases at the beginning of the earliest period presented using a modified retrospective approach. The transition guidance also provides specific guidance for sale and leaseback transactions, build-to-suit leases and amounts previously recognized in accordance with the business combinations guidance for leases. We are currently evaluating our expected adoption method and the impact of this new standard on the financial statements.

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3. Marketable Securities

The following table summarizes the available-for-sale securities held at September 30, 2016 (in thousands):

| | Amortized Cost | Unrealized Gains | Unrealized Losses | Fair Value |
|--|------------------|------------------|-------------------|------------------|
| September 30, 2016 | | | | |
| U.S. government agency securities and treasuries | \$ 25,013 | \$ 7 | \$ (3) | \$ 25,017 |
| Corporate debt securities | 14,695 | — | (12) | 14,683 |
| Total available-for-sale securities | <u>\$ 39,708</u> | <u>\$ 7</u> | <u>\$ (15)</u> | <u>\$ 39,700</u> |

The Company did not have available for sale securities at December 31, 2015. Approximately \$400,000 in available-for-sale securities held as of September 30, 2016 had remaining maturities greater than one year.

4. Fair Value Measurements

The following table sets forth the Company's assets that are measured at fair value on a recurring basis as of September 30, 2016 and December 31, 2015 (in thousands):

| | Total | Quoted prices in active markets (Level 1) | Significant other observable inputs (Level 2) | Significant unobservable inputs (Level 3) |
|---|------------------|---|---|---|
| September 30, 2016 | | | | |
| Assets: | | | | |
| Cash and Cash Equivalents | | | | |
| Money market funds | \$ 23,805 | \$ 23,805 | \$ — | \$ — |
| US government agency securities and treasuries | \$ 1,353 | — | 1,353 | — |
| Corporate debt securities | 1,800 | — | 1,800 | — |
| Total cash and cash equivalents | 26,958 | 23,805 | 3,153 | — |
| Marketable securities | | | | |
| U.S. government agency securities and treasuries | 24,617 | — | 24,617 | — |
| Corporate debt securities | 14,683 | — | 14,683 | — |
| Total marketable securities | 39,300 | — | 39,300 | — |
| U.S. government agency securities and treasuries, long-term | 400 | — | 400 | — |
| Restricted cash | 2,431 | 2,431 | — | — |
| Total assets | <u>\$ 69,089</u> | <u>\$ 26,236</u> | <u>\$ 42,853</u> | <u>\$ —</u> |
| December 31, 2015 | | | | |
| Assets: | | | | |
| Money Market Funds | 89,713 | 89,713 | — | — |
| Total Assets | <u>\$ 89,713</u> | <u>\$ 89,713</u> | <u>\$ —</u> | <u>\$ —</u> |

Cash and cash equivalents

The Company considers all highly liquid securities with original final maturities of three months or less from the date of purchase to be cash equivalents. As of September 30, 2016 and December 31, 2015, cash and cash equivalents are comprised of money market accounts, U.S. government agency securities and corporate debt securities.

Marketable securities

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The cost of available-for-sale securities is adjusted for amortization of premiums and accretion of discounts to maturity. At September 30, 2016 and December 31, 2015, the balance in the Company's accumulated other comprehensive loss was composed solely of activity related to the Company's available-for-sale marketable securities. The Company has not realized material gains or losses on sales of available-for-sale investment securities during any of the periods presented.

As of September 30, 2016, available for sale securities of approximately \$22.9 million were in an unrealized loss position of \$15,000. The Company has the intent and ability to hold such securities until recovery. The Company determined that there were no material changes in the credit risk of the above investments. The Company did not hold any investments with an other-than-temporary impairment as of September 30, 2016 and December 31, 2015.

5. Property and Equipment

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

| | September 30, 2016 | December 31, 2015 |
|------------------------------------|-----------------------|----------------------|
| Construction in Progress | \$ 598 | \$ — |
| Furniture, computers and equipment | 1,540 | 687 |
| Leasehold improvements | 36 | 18 |
| Accumulated depreciation | (340) | (330) |
| | <u>\$ 1,834</u> | <u>\$ 375</u> |

Depreciation expense was approximately \$60,000 and \$12,000 for the three months ended September 30, 2016 and 2015, respectively. Depreciation expense was approximately \$105,000 and \$37,000 for the nine months ended September 30, 2016 and 2015, respectively.

6. Common Stock

The Company's common stock has the following characteristics:

- The holders of shares of common stock are entitled to one vote for each share of common stock held at all meetings of stockholders.
- The holders of shares of common stock are entitled to receive dividends, if and when declared by the Company's board of directors. Since inception, no cash dividends have been declared.

Offerings

In September 2015, the Company entered into a new grant contract with Cancer Prevention and Research Institute of Texas ("CPRIT"), in connection with an award of approximately \$16.8 million. The 2015 award was in the form of an agreement by CPRIT to purchase \$16.8 million of shares of common stock of the Company in a private placement concurrent with the initial public offering of the Company's common stock. On October 5, 2015, CPRIT purchased 2,395,010 shares of the Company's common stock at \$7.00 per share. Net proceeds from the private placement, after related transaction offering costs, were approximately \$16.6 million.

In October 2015, the Company issued 6.25 million shares of common stock in an underwritten public offering, with a price of \$7.00 per share. The underwriters purchased an additional 704,962 shares of common stock pursuant to their option to purchase additional shares. The Company received aggregate net proceeds of approximately \$43.7 million in the public offering, after deducting the underwriting discounts and commissions and estimated offering expenses payable by the Company.

7. Stock Option Plans

2008 Long Term Incentive Plan

During 2008, the Company adopted the 2008 Long Term Incentive Plan, which allows for incentive stock options for its employees and nonqualified stock options (inclusive of restricted stock units and stock appreciation rights) (the “2008 Plan”) for employees and nonemployees under which an aggregate of 330,582 stock options and stock purchase rights may be granted. In December 2013, the total amount available for grant under the 2008 Plan was increased by 224,200 to 554,782. In March 2014, the Company’s board of directors approved an increase of 115,153 shares available for grant pursuant to the 2008 Plan to 669,935. In March 2015, the total amount of available to grant under the 2008 Plan was increased in conjunction with the Company’s offering of Series D preferred stock by 391,650 shares to 1,061,585. Options under the 2008 Plan have a maximum life of 10 years from the date of grant. Options vest at various intervals, as determined by the Company’s board of directors at the date of grant.

2015 Equity Incentive Plan

In August 2015, the Company’s board of directors approved the 2015 Equity Incentive Award Plan (the “2015 Plan”), which was effective in connection with the pricing of the IPO on September 30, 2015. The 2015 Plan provides for the granting of a variety of stock-based compensation awards, including stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, deferred stock awards, dividend equivalent awards, stock payment awards, performance awards and other stock-based awards. The 2015 Plan is the successor to the 2008 Plan and the 764,928 options outstanding in the 2008 Plan at September 30, 2016 may be transferred to the 2015 Plan if awards thereunder terminate, expire or lapse for any reason without the delivery of shares to the holder thereof. Under the 2015 Plan, 1,671,800 shares of the Company’s common stock were initially authorized and reserved for issuance. In March 2016, the Company’s board of directors approved an increase of 1,041,527 shares available for grant pursuant to the 2015 Plan. A combined total of 3,508,492 shares have been authorized and reserved for issuance under the 2008 Plan and 2015 Plan at September 30, 2016.

2015 Employee Stock Purchase Plan

In August 2015, the Company’s board of directors approved the 2015 Employee Stock Purchase Plan (the “ESPP”), which was effective in connection with the pricing of the IPO on September 30, 2015. The ESPP allows eligible employees to purchase shares of the Company’s common stock at a discount through payroll deductions of up to 15% of their eligible compensation, subject to any plan limitations. The ESPP generally provides for set offering periods, and at the end of each offering period, employees are able to purchase shares at 85% of the lower of the fair market value of the Company’s common stock on the first trading day of the offering period or on the last trading day of the offering period. There were no sales under the ESPP as of September 30, 2016. Shares available for future purchase under the ESPP were 375,485 at September 30, 2016.

Stock Option Activity

The Company’s stock option activity for the nine months ended September 30, 2016 was as follows:

| | Number of Shares | Weighted- Average Exercise Price | Weighted-Average Contractual Life (years) |
|---|---------------------|---|---|
| Outstanding at December 31, 2015 | 1,529,459 | 6.29 | 9.00 |
| Granted | 928,250 | 4.41 | |
| Exercised | (5,313) | 1.65 | |
| Forfeited/canceled | (350,432) | 6.85 | |
| Outstanding at September 30, 2016 | <u>2,101,964</u> | <u>\$ 5.37</u> | 8.65 |
| Options exercisable at September 30, 2016 | <u>646,142</u> | <u>\$ 5.33</u> | 7.40 |

Stock Compensation Expense

Total stock-based compensation expense for the three and nine months ended September 30, 2016 was recognized as follows in the statements of comprehensive loss (in thousands):

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|------------------------------------|-------------------------------------|--------|------------------------------------|--------|
| | 2016 | 2015 | 2016 | 2015 |
| Research and development expense | \$ 155 | \$ 57 | \$ 261 | \$ 137 |
| General and administrative expense | 358 | 153 | 941 | 424 |
| Total stock based compensation | \$ 513 | \$ 210 | \$ 1,202 | \$ 561 |

As of September 30, 2016, there was approximately \$5.1 million of unrecognized compensation cost related to the stock options granted under the 2015 Plan, which is expected to be amortized over a weighted-average period of 2.9 years. There were no restricted stock units or stock appreciation rights granted under the 2015 Plan as of September 30, 2016.

8. Income Taxes

The Company has not recorded a provision for income taxes as of September 30, 2016 due to reported net losses since inception.

During the three and nine months ended September 30, 2016 and 2015, the Company had no interest and penalties related to income taxes.

Deferred income taxes reflect the net tax effects of temporary differences between the carrying amount of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The Company has established a valuation allowance due to uncertainties regarding the realization of deferred tax assets based upon the Company's lack of earnings history. The Company files income tax returns in the U.S. federal and Texas jurisdictions. The statute of limitations for assessment by the Internal Revenue Service ("IRS") is open for tax years ending December 31, 2015, 2014, and 2013, although carryforward attributes that were generated for tax years prior to 2013 may still be adjusted upon examination by the IRS if they either have been, or will be, used in a future period. The 2010 and subsequent tax years remain open and subject to examination by the State of Texas. There are currently no federal or state income tax audits in progress.

9. Agreements with Asuragen

On November 3, 2009, the Company entered into an agreement with Asuragen under which Asuragen shares space with and provides services to the Company in support of the Company's business. Such services have included human resources, finance and accounting, information technology, purchasing, shipping and receiving, equipment use, and various facility expenses. The Company paid Asuragen a monthly service fee for the services provided by Asuragen to the Company, which does not include direct charges incurred by Asuragen on behalf of the Company. Total expenses under the Shared Services Agreement with Asuragen totaled approximately \$79,000 and \$100,000 for the three months ended September 30, 2016 and 2015, respectively and \$316,000 and \$295,000 for the nine months ended September 30, 2016 and 2015.

On October 31, 2014, the Company entered into a sublease agreement with Asuragen for use of office, laboratory and shared space. Total rent expense was approximately \$15,000 and \$59,000 for the three and nine months ended September 30, 2016. Both the lease and the shared service agreements expired on August 31, 2016. The Company has entered into a new lease for additional space, as discussed in Note 11.

10. License agreements

Rosetta Genomics Ltd.

In December 2015, the Company entered into a Patent License Agreement (the "License Agreement") with Rosetta Genomics Ltd. ("Rosetta"), licensing to the Company certain patents owned or controlled by Rosetta as specified in the License Agreement. Under the License Agreement, Rosetta granted the Company a non-assignable, non-transferable, worldwide license for certain patents in connection with the development and commercialization of products that relate to the tumor suppressor microRNA MIR-34 ("Products"). This license is exclusive with respect to Products that relate to MRX34, which has been placed on full clinical hold by the Food and Drug Administration ("FDA"), and non-exclusive for products that are not related to MRX34.

Under the License Agreement, the Company paid Rosetta an up-front, non-refundable payment of \$1.6 million, which was accrued as an expense within research and development at December 31, 2015 and subsequently paid in January 2016. The Company is obligated to pay low single-digit royalties on net sales of Products, as well as royalties on sublicense revenues.

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Certain development and regulatory milestone payments totaling \$3 million may also be payable in connection with specified types of Products, upon the achievement of certain development and/ or regulatory milestone events.

Marina Biotech, Inc.

In December 2011, the Company entered into a licensing agreement with Marina Biotech, Inc. (“Marina”), pursuant to which Marina granted to the Company a license to liposomal delivery technology, NOV340, known under the brand name “SMARTICLES,” to develop and commercialize drug products incorporating Marina’s delivery system exclusively in combination with the Company’s first therapeutic product, MRX34, which has been placed on full clinical hold by the FDA. In December 2013, the license agreement was amended to include three additional specific microRNA mimics selected by the Company to use with SMARTICLES on an exclusive basis, and in May 2015, the license agreement was further amended to reduce the amount of a specific milestone payment and to provide for the prepayment of such milestone payment. In August 2015, the Company also entered into a side letter to the license agreement, under which it exercised its right to select an additional specific microRNA as a licensed product, in exchange for the payment of a specified selection fee payment.

The Company has cumulatively paid Marina approximately \$2.1 million through September 30, 2016 in up-front and milestone payments and as consideration for the inclusion within the license of four additional microRNA compounds. As the Company progresses with respect to development and commercialization of its products, the Company will be required to make payments to Marina based upon the achievement of certain development and regulatory milestones, totaling up to \$6 million in the aggregate for each licensed product. The Company has agreed to pay up to an additional \$4 million per licensed product upon the achievement of certain regulatory milestones for a specified number of additional indications, leading to a maximum cap on all milestone payments of \$10 million per product. The exception to this is for the Company’s first therapeutic product candidate, MRX34, where the aggregate of all remaining development and regulatory milestone payments due to Marina, including for all additional indications, is \$4.0 million.

In addition to milestone payments, the Company will be required to pay low single digit royalties on net sales of licensed products other than MRX34, subject to customary reductions and offsets. As a result of the Company’s 2013 amendment to the agreement with Marina, the Company is no longer required to pay a royalty to Marina with respect to sales of the Company’s first therapeutic product candidate, MRX34. If the Company sublicenses its rights under the license from Marina, for each optioned microRNA compound covered by such sublicense the Company is required to pay a specified lump-sum payment representing the remainder of the selection fee for the inclusion of such microRNA compound within the scope of the license agreement, as well as a portion of any revenue the Company receives from such sublicensees at a tiered percentage between the very low single digits and the mid-teens, depending on the circumstances in which the sublicense is entered into.

Yale University

In 2006, Asuragen entered into an exclusive license agreement with Yale University (“Yale”) under certain patent rights relating to microRNAs. This agreement was assigned to the Company by Asuragen in connection with the Company’s acquisition of certain assets, including patent rights, in 2009. In February 2014, the Company as successor-in-interest to Asuragen, amended and restated the exclusive license agreement. Some of the patent filings in the Company’s intellectual property portfolio that are licensed to the Company by Asuragen are also included in the patents licensed under the agreement with Yale. The Company will be required to pay royalties to Yale on net sales of licensed products that contain specified microRNAs, at a percentage ranging from the very low to the low single digits, subject to customary reductions and offsets. The Company will also be required to pay to Yale a portion of specified gross revenue that the Company receives from the Company’s sublicensees at a percentage in the mid-single digits.

The Company will be required to make payments for achievement of certain development and regulatory milestones by products containing one specified microRNA and covered by the licensed patents, of up to \$600,000 in the aggregate for each such product, subject to reduction in certain circumstances. In addition, the Company is required to pay an annual license maintenance fee and minimum annual royalties under certain circumstances. As of September 2016, the Company has suspended all research and development activities.

11. Commitments and Contingencies

Operating Lease

In June 2016, the Company entered into a lease for its corporate headquarters and research facility in Austin, Texas (the “Headquarters”) under an operating lease agreement (the “Lease”). The agreement provides that the lease will commence on the earlier of (i) the date on which the Company first conducts any business in the new Headquarters, (ii) substantial completion of the improvements made to the new Headquarters as defined in the Lease, or (iii) January 1, 2017 (collectively, the “Commencement Date”). The initial term of the lease is for a 123 month period, with the option to extend the lease for up to two consecutive 60 month terms. Rent expense under the Lease for the three and nine months ended September 30, 2016 was approximately \$55,000.

The lease provides annual base rent of approximately \$600,000 in the first year after a three-month rent free period following the Commencement Date, with subsequent annual increases of approximately 3% in the annual base rent. In connection with the lease, the landlord has provided a tenant improvement allowance of approximately \$1.9 million to be used by the Company to build-out certain improvements to the Headquarters. The Lease also provides for an additional improvement allowance of up to \$1.3 million. The additional allowance, if exercised, will amortize over 120 months on a straight line basis. There have been no draws on the additional improvement allowance as of September 30, 2016.

Mima has obtained a standby letter of credit for the initial amount of approximately \$2.4 million, which may be drawn down by the landlord in the event of default. If Mima meets certain requirements, the amount due under the Letter of Credit may be reduced to approximately \$800,000.

Under the Lease agreement, future minimum payments payable are approximately as follows:

| Period ending December 31, | Operating Lease |
|----------------------------|---------------------|
| 2016 (three months) | — |
| 2017 | \$ 450,929 |
| 2018 | 614,855 |
| 2019 | 633,364 |
| 2020 and thereafter | 5,218,227 |
| Total | \$ 6,917,375 |

12. Net Loss Per Share Attributable to Common Stockholders

The following table summarizes the computation of basic and diluted net loss per share attributable to common stockholders of the Company (in thousands, except share and per share data):

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|-------------------------------------|----------------|------------------------------------|-----------------|
| | 2016 | 2015 | 2016 | 2015 |
| Net loss | \$ (5,365) | \$ (6,231) | \$ (17,574) | \$ (16,194) |
| Accretion of convertible preferred stock to redemption value | — | — | — | (448) |
| Accrued dividends on convertible preferred stock | — | (1,554) | — | (3,769) |
| Net loss attributable to common stockholders—basic and diluted | <u>(5,365)</u> | <u>(7,785)</u> | <u>(17,574)</u> | <u>(20,411)</u> |
| Weighted-average number of common shares—basic and diluted | 20,835,868 | 94,753 | 20,832,727 | 91,650 |
| Net loss per share attributable to common stockholders—basic and diluted | \$ (0.26) | \$ (82.16) | \$ (0.84) | \$ (222.70) |

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The following potentially dilutive securities outstanding, prior to the use of the treasury stock method or if-converted method, have been excluded from the computation of diluted weighted-average common shares outstanding, because including them would have had an anti-dilutive effect due to the losses reported.

| | September 30, | |
|-----------------------------|------------------|-------------------|
| | 2016 | 2015 |
| Convertible preferred stock | — | 10,159,614 |
| Stock options | 2,101,964 | 1,528,475 |
| | <u>2,101,964</u> | <u>11,688,089</u> |

As the Company incurred a net loss for the three and nine months ended September 30, 2016 there is no income allocation required under the two-class method or dilution attributed to weighted-average shares outstanding in the computation of diluted loss per share attributable to common stockholders.

13. Subsequent Events

Following the Company's decision to close its Phase 1 study of MRX34, in November 2016 the Company intends to reduce the total number of full-time employees from 36 to 12 to reduce operating expenses and streamline its operations. The Company expects the majority of the employees included in the reduction will be separated from the Company during the fourth quarter of 2016 and expects the remainder will discontinue employment with the Company during the first quarter of 2017. In addition, the Company has entered into retention agreements with certain employees necessary to close the Phase 1 trial of MRX34 and maintain the operations of the Company. The Company estimates that it will incur aggregate cash expenses of approximately \$1.6 million under the workforce reduction, of which \$1.3 million is attributed to one-time severance and benefits payments and \$0.3 million relates to retention payments. In addition, depending on the outcome of the Company's evaluation of strategic alternatives, the Company may recognize an impairment to the carrying value of its fixed assets, additional costs associated with contract terminations, or a combination of both.

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

You should read the following management's discussion and analysis of our financial condition and results of operations in conjunction with our unaudited condensed financial statements and notes thereto included in Part I, Item 1 of this Quarterly Report on Form 10-Q and with our audited financial statements and notes thereto for the year ended December 31, 2015, filed with the U.S. Securities and Exchange Commission, or the SEC, on March 30, 2016.

Special note regarding forward-looking statements

This report contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those discussed in the forward-looking statements. The statements contained in this report that are not purely historical are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act).

Forward-looking statements are often identified by the use of words such as, but not limited to, "anticipate," "believe," "can," "continue," "could," "estimate," "expect," "intend," "may," "plan," "project," "seek," "should," "strategy," "target," "will," "would" and similar expressions or variations intended to identify forward-looking statements. These statements are based on the beliefs and assumptions of our management based on information currently available to management. Such forward-looking statements are subject to risks, uncertainties and other important factors that could cause actual results and the timing of certain events to differ materially from future results expressed or implied by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in the section titled "Risk Factors" included under Part II, Item 1A below. Furthermore, such forward-looking statements speak only as of the date of this report. Except as required by law, we undertake no obligation to update any forward-looking statements to reflect events or circumstances after the date of such statements.

Overview

We are a biopharmaceutical company focused on microRNA-based oncology therapeutics. microRNAs are naturally occurring, short ribonucleic acid, or RNA, molecules, or oligonucleotides, that play a critical role in regulating key biological pathways. Misexpression of even a single microRNA can contribute to disease development and tumor suppressor microRNAs are commonly reduced in cancer. Our scientists and others at leading academic institutions have identified numerous tumor suppressor microRNAs that play key roles in preventing normal cells from becoming cancerous and facilitating proper cancer immunosurveillance. Our mimics of naturally occurring microRNAs are designed to increase this tumor suppressor activity and aid appropriate anti-tumor immune response.

Our first product candidate, MRX34, a mimic of naturally occurring microRNA-34 (miR-34) encapsulated in a liposomal nanoparticle formulation, was the first microRNA mimic to enter clinical development in oncology. MRX34 was studied as a single agent in a multi-center Phase 1 clinical trial, which included patients with primary liver cancer, other solid tumors, and hematological malignancies.

On September 20, 2016, we voluntarily halted the Phase 1 trial following multiple immune-related serious adverse events, or SAEs, observed in patients dosed with MRX34 over the course of the trial. Three of these immune-related events resulted in the patient's death. Subsequently, we received notification from the US Food and Drug Administration, or the FDA, that the Investigational New Drug Application, or IND, for MRX34 is on full clinical hold. Among other comments, the FDA requested a final clinical study report be submitted and noted that a risk-benefit summary with sufficient justification for the continued development of MRX34 would be necessary if we requested removal of the clinical hold.

We have initiated a plan to reduce personnel and expenses to preserve capital and further streamline our operations, and expect to focus operationally on meeting regulatory requirements related to closure of the Phase 1 MRX34 trial and other operating activities consistent with our decision to discontinue development of MRX34. We are evaluating our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company, and have suspended further research and development activities while we evaluate these opportunities.

We were incorporated in 2007 under the laws of Delaware and were maintained as a wholly-owned subsidiary of our former parent company, Asuragen, Inc., or Asuragen, until the end of 2009, when we became an independent entity.

Our operations have historically focused on developing our understanding of and capabilities in microRNA biology, identifying potential product candidates, undertaking preclinical studies, initiating and conducting a clinical trial, protecting and enhancing our intellectual property estate and providing general and administrative support for these activities. Following our reduction in force in November 2016, we are evaluating our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company, and have suspended further research and development activities to reduce operating expenses while we evaluate these opportunities. We have not generated any revenue from product sales and, to date, have funded our operations primarily through the private placements of our capital stock, federal and state government grants and offerings of our equity securities. From our inception through September 30, 2016, we have raised an aggregate of approximately \$167.3 million to fund our operations, of which approximately \$89.9 million was from the issuance of preferred stock for cash and assets, \$48.7 million from a public offering of our common stock, \$16.8 million from a private placement of our common stock and \$11.9 million was from federal and state grants.

Since our inception, we have incurred significant operating losses. Our net loss was \$5.4 million and \$17.6 for the three and nine months ended September 30, 2016. At September 30, 2016, we had an accumulated deficit of \$94.1 million. We expect to continue to incur significant expenses and operating losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and from year to year. We anticipate that our expenses will change significantly as we initiate our reduction in force; suspend our research and development activities; focus on evaluating our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company; and close the Phase 1 clinical trial for MRX34.

Financial Operations Overview

Revenue

We have not generated any revenue from product sales or from collaborations. In the future, we may generate revenue from collaborations and licenses. Revenue may fluctuate from period to period, and the timing and extent of any future revenue will depend on our ability to advance a product candidate, through the clinical trial process and to obtain regulatory approval and our ability, or our future partners' ability, to commercialize our product candidates.

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Research and Development Expenses

Research and development expenses have consisted primarily of costs incurred for our research activities, including our drug discovery efforts, and the development of our product candidates, which have included the following:

- employee-related expenses, including salaries, benefits, travel and stock-based compensation;
- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, or CROs, consultants and our scientific advisory board;
- lab supplies, and acquiring, developing and manufacturing preclinical study materials in accordance with Good Laboratory Practices;
- costs of clinical trials, including costs for management, investigator fees and related vendors that provide services for the clinical trials;
- costs to manufacture the drug used in the clinical trials in accordance with Good Manufacturing Practices;
- license and milestone fees;
- development and prosecution of intellectual property; and
- costs of facilities, depreciation and other expenses.

Research and development costs are expensed as incurred. In certain circumstances, we will make nonrefundable advance payments to purchase goods and services for future use pursuant to contractual arrangements. In those instances, we defer and recognize an expense in the period that we receive or consume the goods or services.

Our research and development expenses have been offset by proceeds derived from federal and state grants. These government grants, which have supplemented our research efforts on specific projects, generally provide for reimbursement of approved costs, as defined in the terms of the grant awards. The proceeds from these reimbursement grants are treated as a reduction to the associated expenses as the allowable expenses are incurred.

At any point in time, we typically had various early stage research and drug discovery projects ongoing. Our internal resources, employees and infrastructure were not directly tied to any one research or drug discovery project and were typically deployed across multiple projects. As such, we did not maintain information regarding the costs incurred for these early stage research and drug discovery programs on a project-specific basis. However, we historically spent the vast majority of our research and development resources on our first product candidate, MRX34, which has been placed on full clinical hold by the FDA.

We anticipate that our research and development expenses will change significantly as we initiate our reduction in force; suspend our research and development activities; focus on evaluating our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company; and close the Phase 1 clinical trial for MRX34.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related benefits, including stock-based compensation, related to our executive, finance and support functions. Other general and administrative expenses include allocated facility-related costs not otherwise included in research and development expenses, travel expenses and professional fees for auditing, tax and legal services. We anticipate that our general and administrative expenses will change significantly as we initiate our reduction in force; suspend our research and development activities; focus on evaluating our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company; and close the Phase 1 clinical trial for MRX34.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate

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our estimates and judgments, including those related to stock-based compensation and clinical trial and pre-clinical study accruals. We base our estimates on historical experience, known trends and events and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. In making estimates and judgments, management employs critical accounting policies. During the nine months ended September 30, 2016, there were no material changes to our critical accounting policies as reported in our Annual Report on Form 10-K for the year ended December 31, 2015, which was filed with the SEC on March 30, 2016.

Results of Operations

Comparison of three months ended September 30, 2016 and 2015:

| | Three Months Ended September 30, | | Dollar Change | % Change |
|--|-------------------------------------|-----------------|------------------|----------------|
| | 2016 | 2015 | | |
| (in thousands) | | | | |
| Statement of operations data: | | | | |
| Operating expenses: | | | | |
| Research and development, before grant reimbursement | \$ 3,391 | \$ 4,927 | \$ (1,536) | (31.2)% |
| Less grant reimbursement | (7) | (244) | 237 | (97.1)% |
| Research and development | 3,384 | 4,683 | (1,299) | (27.7)% |
| General and administrative | 1,940 | 1,556 | 384 | 24.7 % |
| Loss on disposal of assets | 138 | — | 138 | 100.0 % |
| Interest (income) | (97) | (8) | (89) | NM |
| Net loss | <u>\$ 5,365</u> | <u>\$ 6,231</u> | <u>\$ (866)</u> | <u>(13.9)%</u> |

NM- Percentage not meaningful

Research and Development Expenses

Research and development expenses were \$3.4 million for the three months ended September 30, 2016 which was a decrease of \$1.3 million, or 28%, compared to research and development expenses of approximately \$4.7 million for the three months ended September 30, 2015. The decrease in the three months ended September 30, 2016 was primarily due to the following:

- A decrease of approximately \$1.8 million in Phase 1 clinical trial and related costs associated with our first product candidate MRX34, primarily due to upfront drug manufacturing costs incurred in 2015 which were used in clinical trials throughout 2016.
- An offsetting increase of approximately \$220,000 in employee compensation, benefits and stock-based compensation expense due to increased headcount compared to the prior period.

Research and development spending was partially offset by approximately \$7,000 of grant reimbursements for the three months ended September 30, 2016, compared to reimbursement of approximately \$244,000 for the same period in 2015. The decrease is primarily due to two grants which expired in August 2015.

General and Administrative Expenses

General and administrative expenses were approximately \$1.9 million for the three months ended September 30, 2016, which was an increase of approximately \$384,000 or 25%, compared to general and administrative expenses of \$1.6 million for the three months ended September 30, 2015. The increase in the three months ended September 30, 2016 was primarily due to approximately \$339,000 for increased employee compensation, benefits and stock compensation expense primarily due to increased headcount, of which \$134,000 relates to increased payroll and benefits expense and \$205,000 relates to stock-based compensation expense.

Comparison of nine months ended September 30, 2016 and 2015:

| | Nine Months Ended September 30, | | Dollar Change | % Change |
|--|------------------------------------|------------------|------------------|----------|
| | 2016 | 2015 | | |
| (in thousands) | | | | |
| Statement of operations data: | | | | |
| Operating expenses: | | | | |
| Research and development, before grant reimbursement | \$ 11,645 | \$ 13,016 | \$ (1,371) | (10.5)% |
| Less grant reimbursement | (56) | (432) | 376 | (87.0)% |
| Research and development | 11,589 | 12,584 | (995) | (7.9)% |
| General and administrative | 6,119 | 3,618 | 2,501 | 69.1 % |
| Loss on disposal of assets | 138 | — | 138 | 100.0 % |
| Interest (income) | (272) | (8) | (264) | NM |
| Net loss | <u>\$ 17,574</u> | <u>\$ 16,194</u> | <u>\$ 1,380</u> | 8.5 % |

NM- Percentage not meaningful

Research and Development Expenses

Research and development expenses were \$11.6 million for the nine months ended September 30, 2016 which was a decrease of \$1.0 million, or 8%, compared to research and development expenses of approximately \$12.6 million for the nine months ended September 30, 2015. The decrease in the nine months ended September 30, 2016 was primarily due to the following:

- A decrease of approximately \$3.3 million in Phase 1 clinical trials and related costs associated with our first product candidate MRX34, primarily due to upfront drug manufacturing costs incurred in 2015 which were used in clinical trials throughout 2016.
- Offset by an increase of \$1.8 million of increased employee compensation, benefits and stock compensation expense primarily due to increased headcount, of which \$1.7 million related to increased payroll and benefits expense and \$124,000 related to stock based compensation expense.

Research and development spending was partially offset by approximately \$56,000 of grant reimbursements for the nine months ended September 30, 2016, compared to reimbursement of approximately \$432,000 for the same period in 2015. The decrease is primarily due to two grants which expired in August 2015.

General and Administrative Expenses

General and administrative expenses were approximately \$6.1 million for the nine months ended September 30, 2016, which was an increase of approximately \$2.5 million or 69%, compared to general and administrative expenses of \$3.6 million for the nine months ended September 30, 2015. The increase in the nine months ended September 30, 2016 was primarily due to the following:

- Approximately \$1.5 million for additional costs associated with operating as a publicly traded company, including higher legal, audit, insurance, professional and administrative costs.
- Approximately \$1.1 million of increased employee compensation, benefits and stock compensation expense due to increased headcount and changes in compensation, of which \$543,000 related to increased payroll and benefits expense and \$518,000 related to stock based compensation expense.

Liquidity and Capital Resources

Liquidity and Capital Expenditures

Since inception, our operations have been financed primarily through proceeds of \$167.3 million to fund our operations, of which approximately \$89.9 million was from the issuance of preferred stock for cash and assets, \$48.7 million was from a public offering of our common stock, \$16.8 million was from a private placement of our common stock and \$11.9 million was from federal and state grants. At September 30, 2016, we had \$27.0 million of cash and cash equivalents and \$39.7 million invested in marketable securities for a total of \$66.7 million in liquid assets. Our primary uses of cash are to fund operating expenses and evaluate strategic alternatives. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

We believe that our existing cash, cash equivalents and marketable securities as of September 30, 2016, will be sufficient to meet our anticipated cash requirements for at least the next 12 months. However, our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially.

Our future capital requirements are difficult to forecast and will depend on many factors, including:

- our ability to identify and consummate a strategic transaction for the Company;
- the timing and nature of any strategic transactions that we undertake;
- whether we enter into a partnership or business combination;
- our ability to establish and maintain collaboration partnerships, in-license/out-license or other similar arrangements and the financial terms of such agreements;
- the cost incurred in responding to disruptive actions by activist stockholders.

The following table shows a summary of our cash flows for the nine months ended September 30, 2016 and 2015.

| | Nine Months Ended September 30, | |
|---------------------------------|------------------------------------|-------------|
| | 2016 | 2015 |
| | (in thousands) | |
| Net cash provided by (used in): | | |
| Operating activities | \$ (19,232) | \$ (14,519) |
| Investing activities | (43,532) | (187) |
| Financing activities | 9 | 41,090 |
| Net increase (decrease) | \$ (62,755) | \$ 26,384 |

Operating Activities

Net cash used in operating activities was \$19.2 million and \$14.5 million for the nine months ended September 30, 2016 and 2015, respectively. The increase in overall spending for operating activities of approximately \$4.7 million was due to increased headcount and personnel expenses and a reduction in payables and accrued liabilities, primarily related to payments for clinical trial related expenditures.

Investing Activities

Net cash used in investing activities for the periods presented relates primarily to the purchase of marketable securities during the nine months ended September 30, 2016. We invested \$79.8 million in US treasury, US government agency and corporate debt securities with maturities greater than 90 days using surplus proceeds received in connection with our IPO and concurrent private placement in October 2015, partially offset by maturities of debt securities during the period of \$39.9 million. In addition, the Company obtained a standby letter of credit of \$2.4 million in connection with the lease entered into in June 2016 reflected in fiscal year 2016 investing activities as restricted cash.

Financing Activities

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Net cash provided by financing activities was approximately \$41.1 million for the nine months ended September 30, 2015, which was attributable to the initial closing of our offering of Series D convertible preferred stock. There were no financing activities during the nine months ended September 30, 2016.

Contractual Obligations and Commitments

The following table presents payments due under the Company's contractual obligations as of September 30, 2016:

| | Total | Payments Due by Period | | | |
|-----------------|---------------------|------------------------|---------------------|---------------------|---------------------|
| | | Less than 1 Year | 1-3 Years | 3-5 Years | Over 5 Years |
| Operating Lease | \$ 6,917,377 | \$ 300,620 | \$ 1,239,024 | \$ 1,314,585 | \$ 4,063,148 |
| Other | 142,387 | 142,387 | — | — | — |
| | <u>\$ 7,059,764</u> | <u>\$ 443,007</u> | <u>\$ 1,239,024</u> | <u>\$ 1,314,585</u> | <u>\$ 4,063,148</u> |

Off-balance Sheet Arrangements

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

Segment Information

We have one primary business activity and operate as one reportable segment.

JOBS Act

Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other companies.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. At September 30, 2016, we had cash and cash equivalents of \$27.0 million and short-term marketable securities of \$39.7 million consisting of interest-bearing money market funds, U.S. treasury securities, U.S. government agency securities, and corporate debt securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term maturities of our cash equivalents and marketable securities, as well as the low risk profile of our investments, we do not believe a change in interest rates would have a material effect on the fair market value of our cash and cash equivalents and marketable securities.

Item 4. Controls and Procedures.**Evaluation of Disclosure Controls and Procedures**

Our management, with the participation of our chief executive and financial officers, evaluated the effectiveness of our disclosures controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, as of September 30, 2016. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or

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submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate, to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2016, 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 our internal controls over financial reporting during the period covered by this Quarterly Report on Form 10-Q identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II-OTHER INFORMATION

Item 1. Legal Proceedings

We are not currently a party to any material legal proceedings. We may at times be involved in litigation and other legal claims in the ordinary course of business. When appropriate in management's estimation, we may record reserves in our financial statements for pending litigation and other claims.

Item 1A. Risk Factors

Our business involves a high degree of risk. You should consider carefully the following risks, together with all the other information in this periodic report, including our financial statements and notes thereto and "Management's Discussion and Analysis of Financial Condition and Results of Operations." If any of the following risks actually materializes, our operating results, financial condition and liquidity could be materially adversely affected. As a result, the trading price of our common stock could decline. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations.

Risk Factors

Risks Related to Our Evaluation of Strategic Alternatives

Our activities to evaluate and pursue strategic alternatives may not be successful.

In September 2016, we voluntarily halted the Phase 1 trial following multiple immune-related serious adverse events, or SAEs, observed in patients dosed with MRX34 over the course of the trial. Three of these immune-related events resulted in the patient's death. Subsequently, we received notification from the U.S. Food and Drug Administration, or the FDA, that the Investigational New Drug Application, or IND, for MRX34 is on full clinical hold. Following the close of our Phase 1 clinical trial of MRX34, we are evaluating our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company, and we have suspended further research and development activities to reduce operating expenses while we evaluate these opportunities. We expect to devote significant time and resources to identifying and evaluating a strategic transaction, however, there can be no assurance that such activities will result in any agreements or transactions that will enhance shareholder value. Further, any strategic transaction that is completed ultimately may not deliver the anticipated benefits or enhance shareholder value.

Any such strategic transaction may require us to incur non-recurring or other charges, may increase our near- and long-term expenditures and may pose significant integration challenges or disrupt our management or business, which could adversely affect our operations and financial results. For example, these transactions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;
- incurrence of substantial debt or dilutive issuances of equity securities to pay for acquisitions;
- higher-than-expected acquisition and integration costs;
- write-downs of assets or goodwill or impairment charges;
- increased amortization expenses;
- difficulty and cost in combining the operations and personnel of any acquired businesses with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and
- inability to retain key employees of our company or any acquired businesses.

Accordingly, although there can be no assurance that we will undertake or successfully complete any strategic transactions of the nature described above, any transactions that we do complete may be subject to the foregoing or other risks, could have a material adverse effect on our business, financial condition and prospects.

If we do not successfully consummate a strategic transaction, our board of directors may decide to pursue a dissolution and liquidation of our company. In such an event, the amount of cash available for distribution to our stockholders will depend

heavily on the timing of such liquidation as well as the amount of cash that will need to be reserved for commitments and contingent liabilities.

There can be no assurance that the process to identify a strategic transaction will result in a successfully consummated transaction. If no transaction is completed, our board of directors may decide to pursue a dissolution and liquidation of our company. In such an event, the amount of cash available for distribution to our stockholders will depend heavily on the timing of such decision and, ultimately, such liquidation, since the amount of cash available for distribution continues to decrease as we fund our operations while we evaluate our strategic alternatives. In addition, if our board of directors were to approve and recommend, and our stockholders were to approve, a dissolution and liquidation of our company, we would be required under Delaware corporate law to pay our outstanding obligations, as well as to make reasonable provision for contingent and unknown obligations, prior to making any distributions in liquidation to our stockholders. Our commitments and contingent liabilities may include (i) regulatory and clinical obligations remaining under our Phase 1 trial for MRX34; (ii) obligations under our employment and related agreements with certain employees that provide for severance and other payments following a termination of employment occurring for various reasons, including a change in control of our company; (iii) potential litigation against us, and other various claims and legal actions arising in the ordinary course of business; and (iv) non-cancelable facility lease obligations. As a result of this requirement, a portion of our assets may need to be reserved pending the resolution of such obligations. In addition, we may be subject to litigation or other claims related to a dissolution and liquidation of our company. If a dissolution and liquidation were pursued, our board of directors, in consultation with its advisors, would need to evaluate these matters and make a determination about a reasonable amount to reserve. Accordingly, holders of our common stock could lose all or a significant portion of their investment in the event of a liquidation, dissolution or winding up of our company.

Our business to date has been almost entirely dependent on the success of MRX34, and we have recently decided to discontinue further development of MRX34 and devote significant time and resources to identifying and evaluating strategic alternatives, which may not be successful.

To date, we have invested substantially all of our efforts and financial resources in the research and development of MRX34, which was our only product candidate to enter in clinical trials. On September 20, 2016, we voluntarily halted the Phase 1 trial following multiple immune-related serious adverse events, or SAEs, observed in patients dosed with MRX34 over the course of the trial. Three of these immune-related events resulted in the patient's death. Subsequently, we received notification from the U.S. Food and Drug Administration, or the FDA, that the Investigational New Drug Application, or IND, for MRX34 is on full clinical hold. Among other comments, the FDA requested a final clinical study report be submitted and noted that a risk-benefit summary with sufficient justification for the continued development of MRX34 would be necessary if we requested removal of the clinical hold.

We have initiated a plan to reduce personnel and expenses to preserve capital and further streamline our operations, and expect to focus operationally on meeting regulatory requirements related to the closure of the Phase 1 MRX34 trial and other operating activities consistent with our decision to discontinue development of MRX34. We are evaluating strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company, and have suspended further research and development activities to reduce operating expenses while we evaluate these opportunities.

There can be no assurance that our process to identify and evaluate potential strategic alternatives will result in any definitive offer to consummate a strategic transaction, or if made, what the terms thereof will be or that any transaction will be approved or consummated. If any definitive offer to consummate a strategic transaction is received, there can be no assurance that a definitive agreement will be executed or that, if a definitive agreement is executed, the transaction will be consummated. In addition, there can be no assurance that any transaction, involving our company and/or assets, that is consummated would enhance shareholder value. There also can be no assurance that we will conduct further drug research or development activities in the future.

We are substantially dependent on our remaining employees to facilitate the consummation of a strategic transaction.

Our ability to successfully complete a strategic transaction depends in large part on our ability to retain certain of our remaining personnel, particularly Paul Lammers, M.D., M.Sc., our president and chief executive officer. Despite our efforts to retain these employees, one or more may terminate their employment with us on short notice. The loss of the services of any of these employees could potentially harm our ability to evaluate and pursue strategic alternatives, as well as fulfill our reporting obligations as a public company.

Risks Related to Our Limited Operating History, Financial Position and Capital Requirements

We have incurred significant losses since inception. We anticipate that we will continue to incur significant losses for the foreseeable future, and if we are unable to achieve and sustain profitability, the market value of our common stock will likely decline.

We are a biopharmaceutical company with a limited operating history. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We have not generated any product revenues and we do not expect to generate any product revenues for the foreseeable future. We have incurred losses in each year since our founding in 2007 and we expect to continue to incur significant operating losses for the foreseeable future. The amount of future losses is uncertain. None of our product candidates has been approved for sale. We have historically devoted substantially all of our efforts to research and development, including our preclinical and nonclinical development activities. Following the close of our Phase 1 clinical trial of MRX34, we are evaluating strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company, and have suspended further research and development activities to reduce operating expenses while we evaluate these opportunities. To date, we have derived all of our funding from our collaboration with our former parent company, Asuragen, Inc., or Asuragen, private placements of our capital stock and government grants for research and development. Our net loss for the nine months ended September 30, 2016 was \$17.6 million. Since inception, we have incurred net losses leading to an accumulated deficit of approximately \$94.1 million as of September 30, 2016.

We expect to continue to incur significant expenses and operating losses for the foreseeable future as we evaluate strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital. If we are unable to achieve and sustain profitability, the market value of our common stock will likely decline. Because of the numerous risks and uncertainties associated with developing biopharmaceutical products, we are unable to predict the extent of any future losses or whether we will become profitable.

Our short operating history may make it difficult to evaluate the success of our business to date and to assess our future viability.

We are a biopharmaceutical company that was founded in 2007 and did not exist as a standalone company until 2009. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying and evaluating potential product candidates and delivery technologies, undertaking nonclinical studies, filing an IND application with the FDA, and conducting a Phase 1 clinical trial of MRX34, our first product candidate. Except for MRX34 (which is currently on full clinical hold), none of our product candidates are in clinical development. We have not demonstrated our ability to initiate clinical trials for product candidates other than MRX34, or successfully complete any clinical trials, including large-scale, pivotal clinical trials, obtain marketing approvals, manufacture a commercial scale medicine, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Typically, it takes many years to develop one new product candidate from the time it is discovered to when it is available for treating patients. Consequently, any predictions about our future success or viability, or any evaluation of our business or prospects, may not be as accurate as they could be if we had a longer operating history. In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown challenges.

Although we have suspended our research and development activities, if we resume the development of any of our product candidates, we would need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or terminate our product development, other operations or commercialization efforts.

Developing biopharmaceutical products, including conducting preclinical and nonclinical studies and clinical trials, is an expensive and highly uncertain process that takes years to complete. Our expenses may increase substantially if we conduct research and development of any of our product candidates or pursue marketing approval for any such product candidates in the future. Additional clinical trials, including one or more late-stage pivotal trials, would be required to obtain potential marketing approval for any product candidates we may decide to pursue or develop.

As of September 30, 2016, we had working capital of \$64.0 million, primarily comprised of cash and cash equivalents of \$27.0 million and marketable securities of \$39.3 million. Based on our current operating plan, we believe that our available cash, cash equivalents and marketable securities at such date are sufficient to fund our anticipated levels of operation for at least the next 12 months. Our future capital requirements for the period for which we expect our existing resources to support our operations may vary significantly from what we expect. Our funds at September 30, 2016 will not be sufficient to obtain marketing approval for any of our product candidates. As a result, if we resume the development of any of our product candidates, we would be required to obtain additional financing in the future, which we may obtain through public or private

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equity offerings, debt financings, credit facilities, government grants and contracts and/or strategic collaborations. If we are required to secure additional capital, such additional fundraising efforts may divert our management from our day-to-day activities, which may adversely affect our ability to develop and commercialize future product candidates. We believe our ability to raise additional funds is impaired as a result of the current full clinical hold on our IND for MRX34. Additional financing may not be available to us when we need it or it may not be available to us on favorable terms, if at all. If we are unable to obtain adequate financing or form favorable collaborations, when needed, we may have to delay, reduce the scope of or eliminate one or more of our clinical trials, research and development programs or our commercialization efforts.

Additionally, our future financing requirements will depend on many factors, some of which are beyond our control, including:

- our ability to identify and consummate a strategic transaction for the Company;
- the timing and nature of any strategic transactions that we undertake;
- whether we enter into a partnership or business combination;
- our ability to establish and maintain collaboration partnerships, in-license/out-license or other similar arrangements and the financial terms of such agreements;
- the cost incurred in responding to disruptive actions by activist stockholders

Future capital requirements would also depend on the extent to which we acquire or invest in additional complementary businesses, products and technologies. We currently have no understandings, commitments or agreements relating to any of these types of transactions.

Although we have suspended our research and development activities, if we resume the development of any of our product candidates, raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance additional cash needs, if any, through public or private equity offerings, debt financings, credit facilities, government grants and contracts and/or strategic collaborations.

Although we have suspended our research and development activities, if we resume the development of any of our product candidates, to raise capital, we may from time to time issue additional shares of common stock at a discount from the then-current trading price of our common stock. As a result, our common stockholders would experience immediate dilution upon the purchase of any shares of our common stock sold at such discount. In addition, as opportunities present themselves, we may enter into financing or similar arrangements in the future, including the issuance of debt securities, preferred stock or common stock. Whether or not we issue additional shares of common stock at a discount, any issuance of common stock will, and any issuance of other equity securities, securities convertible into equity securities or options, warrants or other rights to purchase equity securities, result in additional dilution of the percentage ownership of our existing stockholders and could cause our stock price to decline. New investors could also gain rights, preferences and privileges senior to those of holders of our common stock, which could cause the price of our common stock to decline. Debt securities may also contain covenants that restrict our operational flexibility, impose liens or other restrictions on our assets, restrict our ability to incur additional debt, impose limitations on our ability to acquire, sell or license intellectual property or impose other operating restrictions that could adversely affect our business and could also cause the price of our common stock to decline.

Other than our collaboration with our former parent company, Asuragen, private placements of our capital stock, and public offerings of common stock, the only significant external source of funds to date has been state and federal government grants for research and development. The grants have been, and any future government grants and contracts we may receive may be, subject to the risks and contingencies set forth below under the risk factor entitled “Reliance on government funding for our programs may add uncertainty to our research and commercialization efforts with respect to those programs that are tied to such funding and may impose requirements that limit our ability to take certain actions, increase the costs of commercialization and production of product candidates developed under those programs and subject us to potential financial penalties, which could materially and adversely affect our business, financial condition and results of operations.” Although we might apply for government and private contracts and grants in the future, we cannot guarantee that we will be successful in obtaining additional grants or contracts for any of our product candidates or programs.

Risks Related to Product Development and Commercialization

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Although we have suspended our research and development activities, if we resume development of any of our product candidates, the approach we have previously taken to discover and develop novel therapeutics using microRNA is unproven and may never lead to marketable products.

The scientific discoveries that form the basis for our prior efforts to discover and develop new drugs are relatively recent. To date, neither we nor any other company has received regulatory approval to market therapeutics utilizing microRNA. The scientific evidence to support the feasibility of developing drugs based on these discoveries is both preliminary and limited. Successful development of microRNA-based products by us would require solving a number of issues, including providing suitable methods of stabilizing the microRNA material and delivering it into target cells in the human body. In addition, if we resume the research and development of any compounds, such compounds may not demonstrate in patients the chemical and pharmacological properties ascribed to them in laboratory and nonclinical studies, and they may interact with human biological systems in unforeseen, ineffective or even harmful ways.

Further, the FDA has relatively limited experience with microRNA-based therapeutics. No regulatory authority has granted approval to any person or entity, including us, to market or commercialize microRNA therapeutics, which may increase the complexity, uncertainty and length of the regulatory approval process for our product candidates. If our microRNA technologies prove to be ineffective, unsafe or commercially unviable, our entire pipeline would have little, if any, value, which would have a material adverse effect on our business, financial condition, results of operations and prospects.

Further, our exclusive focus on microRNA technology for developing products as opposed to multiple, more proven technologies for drug development increases the risk associated with our business. If we are not successful in developing a product candidate using microRNA technology, we may not be able to identify and successfully implement an alternative product development strategy.

Although we have suspended our research and development activities, if we resume development of any of our product candidates they may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

As with many pharmaceutical products and product candidates, our potential product candidates may produce undesirable side effects or adverse reactions or events. For example, on September 20, 2016, we announced our decision to close the Phase 1 study of MRX34 and we voluntarily halted enrollment and dosing in the clinical study following multiple immune-related SAEs observed in patients dosed with MRX34 over the course of the trial. Three of these immune-related SAEs resulted in the patient's death. Following the close of our Phase 1 clinical trial of MRX34, we are evaluating our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company, and have suspended further research and development activities to reduce operating expenses while we evaluate these opportunities.

Although we have suspended our research and development activities, if we or others resume the research and development of our product candidates and we or others identify undesirable side effects caused by one of our product candidates, any of the following adverse events could occur:

- we may be required, or we may decide, to halt or delay further clinical development of such other our product candidates;
- the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all indications; or
- product-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims.

Although we have suspended our research and development activities, if we or others resume the research and development of our potential product candidates and any such candidates are approved, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw their approval of the product;
- we or others may be required to recall a product or change the way such product is administered to patients;

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- additional restrictions may be imposed on the marketing of the particular product or the manufacturing processes for the product or any component thereof;
- regulatory authorities may require the addition of labeling statements, such as a “black box” warning or a contraindication;
- we or others may be required to implement a REMS or create a Medication Guide outlining the risks of such side effects for distribution to patients;
- we or others could be sued and held liable for harm caused to patients;
- the product may become less competitive; and
- our reputation may suffer.

If we or others resume the research and development of our potential product candidates, any of the foregoing events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, which could materially and adversely affect our results of operations and business.

We face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use or misuse of our product candidates harms patients, or is perceived to harm patients even when such harm is unrelated to our product candidates, we could be subject to costly and damaging product liability claims. If we are unable to obtain adequate insurance or are required to pay for liabilities resulting from a claim excluded from, or beyond the limits of, our insurance coverage, a material liability claim could adversely affect our financial condition.

The use or misuse of our product candidates in clinical trials exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. Certain oligonucleotide therapeutics and liposomal drug delivery products have shown injection site reactions, infusion reactions, and pro-inflammatory effects, and may also lead to organ dysfunction, including impairment of kidney or liver function. There is a risk that our product candidates may induce similar adverse events. Patients with the diseases targeted by our product candidates are often already in severe and advanced stages of disease and have both known and unknown significant pre-existing and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may be related to our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to our products, the investigation into the circumstance may be time-consuming or inconclusive. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

Although we have product liability insurance that we feel is appropriate for our stage of development, which covers our clinical trials in the United States, for up to \$1 million per occurrence, up to an aggregate limit of \$5 million, our insurance may be insufficient to reimburse us for any expenses or losses we may suffer. We have obtained an additional product liability insurance policy for our clinical trials completed in the Republic of Korea. We do not know whether we will be able to continue to obtain product liability coverage and obtain expanded coverage if we require it, in sufficient amounts to protect us against losses due to liability, on acceptable terms, or at all. We may not have sufficient resources to pay for any liabilities resulting from a claim excluded from, or beyond the limits of, our insurance coverage. Where we have provided indemnities in favor of third parties under our agreements with them, there is also a risk that these third parties could incur liability and bring a claim under such indemnities. An individual may bring a product liability claim against us alleging that one of our product candidates or products causes, or is claimed to have caused, an injury or is found to be unsuitable for consumer use. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. Any product liability claim brought against us, with or without merit, could result in:

- initiation of investigations by regulators;
- substantial costs of litigation, including monetary awards to patients or other claimants;
- liabilities that substantially exceed our product liability insurance, which we would then be required to pay ourselves;

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- an increase in our product liability insurance rates or the inability to maintain insurance coverage in the future on acceptable terms, if at all;
- the diversion of management's attention from our business; and
- damage to our reputation and the reputation of our products and our technology.

Product liability claims may subject us to the foregoing and other risks, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Our Reliance on Third Parties

If we attempt to form collaborations in the future with respect to our product candidates, we may not be able to do so.

We may attempt to form strategic alliances, create joint ventures or collaborations or enter into licensing arrangements with third parties with respect to our programs that we believe will complement or augment our existing business. We may face significant competition in seeking appropriate strategic partners, and the negotiation process to secure appropriate terms is time-consuming and complex. We may not be successful in our efforts to establish such a strategic partnership for any product candidates and programs on terms that are acceptable to us, or at all. This may be because our product candidates and programs may be deemed to be at too early of a stage of development for collaborative effort, our research and development pipeline may be viewed as insufficient, the competitive or intellectual property landscape may be viewed as too intense or risky, and/or third parties may not view our product candidates and programs as having sufficient potential for commercialization, including the likelihood of an adequate safety and efficacy profile.

If we entered into a collaboration, we may be unable to realize the potential benefits of any collaboration.

If we enter into a collaboration with respect to the development and/or commercialization of one or more product candidates, there is no guarantee that the collaboration would be successful. Collaborations may pose a number of risks, including:

- collaborators often have significant discretion in determining the efforts and resources that they will apply to the collaboration, and may not commit sufficient resources to the development, marketing or commercialization of the product or products that are subject to the collaboration;
- collaborators may not perform their obligations as expected;
- any such collaboration may require us to relinquish potentially valuable rights to our current product candidates, potential products or proprietary technologies or grant licenses on terms that are not favorable to us;
- collaborators may cease to devote resources to the development or commercialization of our product candidates if the collaborators view our product candidates as competitive with their own products or product candidates;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the course of development, might cause delays or termination of the development or commercialization of product candidates, and might result in legal proceedings, which would be time-consuming, distracting and expensive;
- collaborators may be impacted by changes in their strategic focus or available funding, or business combinations involving them, which could cause them to divert resources away from the collaboration;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- the collaborations may not result in us achieving revenues to justify such transactions; and
- collaborations may be terminated and, if terminated, may result in a need for us to raise additional capital to resume further development or commercialization of the applicable product candidate.

As a result, a collaboration may not result in the successful development or commercialization of our product candidates.

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Reliance on government funding for our programs may add uncertainty to our research and commercialization efforts with respect to those programs that are tied to such funding and may impose requirements that limit our ability to take certain actions, and subject us to potential financial penalties, which could materially and adversely affect our business, financial condition and results of operations.

During the course of our development of our product candidates, we have been funded in significant part through federal and state grants, including but not limited to the substantial funding we have received from the Texas Emerging Technology Fund and the Cancer Prevention and Research Institute of Texas, or CPRIT. In addition to the funding we have received to date, we have in the past applied for federal and state grants to receive additional funding. Contracts and grants funded by the U.S. government, state governments and their related agencies, including our contracts with the State of Texas pertaining to funds we have already received, include provisions that reflect the government’s substantial rights and remedies, many of which are not typically found in commercial contracts, including powers of the government to:

- require repayment of all or a portion of the grant proceeds, in certain cases with interest, in the event we violate certain covenants pertaining to various matters that include any potential relocation outside of the State of Texas, failure to achieve certain milestones or to comply with terms relating to use of grant proceeds, or failure to comply with certain laws;
- terminate agreements, in whole or in part, for any reason or no reason;
- reduce or modify the government’s obligations under such agreements without the consent of the other party;
- claim rights, including intellectual property rights, in products and data developed under such agreements;
- audit contract-related costs and fees, including allocated indirect costs;
- suspend the contractor or grantee from receiving new contracts pending resolution of alleged violations of procurement laws or regulations;
- impose U.S. manufacturing requirements for products that embody inventions conceived or first reduced to practice under such agreements;
- impose qualifications for the engagement of manufacturers, suppliers and other contractors as well as other criteria for reimbursements;
- suspend or debar the contractor or grantee from doing future business with the government;
- control and potentially prohibit the export of products;
- pursue criminal or civil remedies under the False Claims Act, False Statements Act and similar remedy provisions specific to government agreements; and
- limit the government’s financial liability to amounts appropriated by the U.S. Congress on a fiscal-year basis, thereby leaving some uncertainty about the future availability of funding for a program even after it has been funded for an initial period.

In addition to those powers set forth above, the government funding we may receive could also impose requirements to make payments based upon sales of our products in the future, if any. For example, under the terms of our 2010 award from CPRIT, we are required to pay CPRIT a portion of our revenues from sales of certain products by us, or received from our licensees or sublicensees, at a percentage in the low single digits until the aggregate amount of such payments equals a specified multiple of the grant amount, and thereafter at a rate of less than one percent, subject to our right, under certain circumstances, to make a one-time payment in a specified amount to CPRIT to buy out such payment obligations. See also “Business — Strategic Partnerships and Licenses” of Part I, Item 1 of our Annual Report for the year ended December 31, 2015 for a description of this CPRIT agreement, which includes a description of our obligations to make royalty payments.

We may not have the right to prohibit the U.S. government from using certain technologies developed by us, and we may not be able to prohibit third-party companies, including our competitors, from using those technologies in providing products and services to the U.S. government. The U.S. government generally takes the position that it has the right to royalty-free use of technologies that are developed under U.S. government contracts. These and other provisions of government grants may also apply to intellectual property we license now or in the future.

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In addition, government contracts and grants normally contain additional requirements that may increase our costs of doing business, reduce our profits, and expose us to liability for failure to comply with these terms and conditions. These requirements include, for example:

- specialized accounting systems unique to government contracts and grants;
- mandatory financial audits and potential liability for price adjustments or recoupment of government funds after such funds have been spent;
- public disclosures of certain contract and grant information, which may enable competitors to gain insights into our research program; and
- mandatory socioeconomic compliance requirements, including labor standards, non-discrimination and affirmative action programs and environmental compliance requirements.

If we fail to maintain compliance with any such requirements that may apply to us now or in the future, we may be subject to potential liability and to termination of our contracts.

Our business involves the use of hazardous materials and we and our third-party manufacturers must comply with environmental laws and regulations, which may be expensive and restrict how we do business.

Our third-party manufacturers' activities and our own activities involve the controlled storage, use and disposal of hazardous materials, including the components of our pharmaceutical product candidates, test samples and reagents, biological materials and other hazardous compounds. We and our manufacturers are subject to federal, state, local and foreign laws and regulations governing the use, generation, manufacture, storage, handling and disposal of these hazardous materials. We currently carry no insurance specifically covering environmental claims relating to the use of hazardous materials. Although we believe that our safety procedures for handling and disposing of these materials and waste products comply with the standards prescribed by these laws and regulations, we cannot eliminate the risk of accidental injury or contamination from the use, storage, handling or disposal of hazardous materials. In the event of an accident, state or federal or other applicable authorities may curtail our use of these materials and/or interrupt our business operations. In addition, if an accident or environmental discharge occurs, or if we discover contamination caused by prior operations, including by prior owners and operators of properties we acquire, we could be liable for cleanup obligations, damages and fines. If such unexpected costs are substantial, this could significantly harm our financial condition and results of operations.

Risks Related to Administrative Operations

Recent changes in our executive leadership and any similar changes in the future may serve as a significant distraction for our management and employees.

Since the beginning of 2016, there have been three changes to our executive leadership team. In May 2016, we transitioned our Chief Medical Officer from Dr. Sinil Kim to Dr. Vincent O'Neill and, in June 2016, we mutually agreed with Dr. Miguel Barbosa that Dr. Barbosa would resign as our Chief Scientific Officer. In November 2016, we terminated the employment of Jon Irvin, our Vice President of Finance, in connection with our reduction in force as part of a plan to reduce operating costs. Such changes, or any other future changes in our executive leadership, may disrupt our operations as we adjust to the reallocation of responsibilities and assimilate new leadership and, potentially, differing perspectives on our strategic direction. If the transition in executive leadership is not smooth, the resulting disruption could negatively affect our evaluation of strategic alternatives.

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

Despite the implementation of security measures, our internal computer systems and those of our CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such 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 programs. For example, the loss of clinical trial data from completed or ongoing clinical trials for any of our product candidates 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 results in a loss of or damage to our data or applications, or

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inappropriate disclosure of confidential or proprietary information, including the confidential medical information of clinical trial participants, we could incur liability.

Our employees, independent contractors, principal investigators, CROs, consultants and vendors, if any, may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk that our employees, independent contractors, principal investigators, CROs, consultants and vendors, if any, may engage in fraudulent or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates: (i) FDA regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA; (ii) manufacturing standards; (iii) federal and state healthcare fraud and abuse laws and regulations; or (iv) laws that require the true, complete and accurate information or data. Specifically, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct by our employees and other third parties, 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 governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Requirements associated with being a public company have increased and will continue to increase our costs significantly, as well as divert significant company resources and management attention.

Prior to our initial public offering in 2015, we were not subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, or the other rules and regulations of the Securities and Exchange Commission, or SEC, or any securities exchange relating to public companies. We are working with our legal, independent accounting and financial advisors to identify those areas in which changes should be made to our financial and management control systems to manage our growth and our obligations as a public company. These areas include corporate governance, corporate control, disclosure controls and procedures and financial reporting and accounting systems. We have made, and will continue to make, changes in these and other areas. However, the expenses associated with operating as a public company are, material, particularly after we cease to be an “emerging growth company.” Compliance with the various reporting and other requirements applicable to public companies also requires considerable time and attention of management. In addition, the changes we have made, and continue to make, may not be sufficient to allow us to satisfy our obligations as a public company on a timely basis, or at all.

However, for as long as we remain an “emerging growth company” as defined in the Jumpstart our Business Startups Act, or the JOBS Act, we may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not “emerging growth companies,” including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. Because the JOBS Act has only recently been enacted, it is not yet clear whether investors will accept the more limited disclosure requirements that we may be entitled to follow while we are an “emerging growth company.” If they do not, we elect to comply with disclosure requirements as if we were not an “emerging growth company,” in which case we would incur the greater expenses associated with such disclosure requirements.

We will remain an “emerging growth company” for up to five years after the completion of our initial public offering, although if the market value of our common stock that is held by non-affiliates exceeds \$700 million as of any June 30 before that time or if we have total annual gross revenues of \$1 billion or more during any fiscal year before that time, we would cease to be an “emerging growth company” as of the end of that fiscal year, or if we issue more than \$1 billion in non-convertible debt in a three-year period, we would cease to be an “emerging growth company” immediately.

In addition, being a public company could make it more difficult or costly for us to obtain certain types of insurance, including directors’ and officers’ liability insurance, and we may be forced to accept reduced policy limits and coverage or

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incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

If we are not able to implement the requirements of Section 404 of the Sarbanes-Oxley Act of 2002 in a timely manner or with adequate compliance, we may be subject to sanctions by regulatory authorities.

Section 404 of the Sarbanes-Oxley Act of 2002 requires that we evaluate and determine the effectiveness of our internal controls over financial reporting and, beginning with our annual report for fiscal year 2016, provide a management report on the internal control over financial reporting. If we have a material weakness in our internal control over financial reporting, we may not detect errors on a timely basis and our financial statements may be materially misstated. We will be evaluating our internal controls systems to allow management to report on, and eventually allow our independent auditors to attest to, our internal controls. We will be performing the system and process evaluation and testing (and any necessary remediation) required to comply with the management certification and eventual auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002. The aforementioned auditor attestation requirements will not apply to us until we are no longer considered an “emerging growth company.”

To date, we have never conducted a review of our internal controls for the purpose of providing the reports required by these rules. We cannot be certain as to the timing of completion of our evaluation, testing and remediation actions or the impact of the same on our operations. If we are not able to implement the requirements of Section 404 in a timely manner or with adequate compliance, we may be subject to sanctions or investigation by regulatory authorities, such as the SEC or The NASDAQ Stock Market LLC, or NASDAQ. Any such action could adversely affect our financial results or investors’ confidence in us and could cause our stock price to fall. Moreover, if we are not able to comply with the requirements of Section 404 in a timely manner, or if we or our independent registered public accounting firm identifies deficiencies in our internal controls that are deemed to be material weaknesses, we could be subject to sanctions or investigations by the SEC, NASDAQ or other regulatory authorities, which would entail expenditure of additional financial and management resources and could materially adversely affect our stock price. Deficient internal controls could also cause us to fail to meet our reporting obligations or cause investors to lose confidence in our reported financial information, which could have a negative effect on our stock price.

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

We have incurred substantial losses during our history and do not expect to become profitable in 2016 and may never achieve profitability. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire. We may be unable to use these losses to offset income before such unused losses expire. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an “ownership change,” generally defined as a greater than 50 percentage point change (by value) in its equity ownership over a three-year period, the corporation’s ability to use its pre-change net operating loss, or NOL, carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be further limited. We believe that we have experienced at least one ownership change in the past. We may also experience additional ownership changes as a result of subsequent shifts in our stock ownership, including as a result of our initial public offering. Accordingly, our ability to use our pre-change NOL carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. For these reasons, we may not be able to utilize any or a material portion of our NOL carryforwards and other tax attributes.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations, and have a material adverse effect on our business, financial condition and results of operations. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our enterprise financial systems or manufacturing resource planning and enterprise quality systems, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Furthermore, certain integral parties in our supply chain are geographically concentrated and operating from single sites, increasing their vulnerability to natural disasters or other sudden, unforeseen and SAEs. Although we believe there to be

sufficient alternative suppliers in other geographic locations, if such an event were to affect such existing parties in our supply chain, it could have a material adverse effect on our business.

Risks Related to Intellectual Property

If we resume the research and development of our product candidates despite suspending such activities and are unable to obtain and maintain sufficient patent protection for our technology and product candidates, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and the value of our product candidates may be adversely affected.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our technologies.

In particular, our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our product candidates. If we do not adequately protect our intellectual property, competitors may be able to use our technologies and erode or negate any competitive advantage we may have, which could harm the potential value of our product candidates. To protect our proprietary position, we file patent applications in the United States and in limited jurisdictions abroad related to our product candidates and compounds in development that may become our product candidates. The patent application and approval process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. We may also fail to identify patentable aspects of our research and development, if we resume our research and development of our product candidates, before it is too late to obtain patent protection.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. This uncertainty includes changes to the patent laws through either legislative action to change statutory patent law or court action that may reinterpret existing law in ways affecting the scope or validity of issued patents. The patent applications that we own or in-license may fail to result in issued patents in the United States or in foreign countries in which we pursue protection with claims that cover our product candidates. 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 have issued, or do successfully issue, from patent applications that we own or license, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. For example, patents granted by the European Patent Office, or EPO, may be challenged, also known as opposed, by any person within nine months from the publication of their grant. In May 2015, two separate and unidentified parties filed submissions before the EPO opposing a granted European patent related to MRX34, EP2302055 (the '055 Patent), in-licensed to us from Asuragen. We have reviewed these submissions and have submitted our response. All of the claims of the '055 Patent remain valid and in force during the opposition proceedings. It is not possible to predict the outcome of the opposition proceedings, for example whether the patent will be maintained, limited in scope or whether the grant may be revoked. If the '055 Patent is ultimately narrowed in scope or revoked during the opposition proceedings, the patent protection afforded by the '055 Patent. Even if they are unchallenged, our patents may not adequately protect our product candidates, provide any competitive advantage or prevent others from designing around our claims. If the breadth or strength of protection provided by the patents and patent applications we hold, in-license or pursue with respect to our product candidates is threatened or insufficient, it could dissuade companies from collaborating with us to develop our product candidates and could have a material adverse effect on our business, financial condition, results of operations and prospects.

Currently, our patent portfolio includes over 10 issued U.S. patents and over 42 pending U.S. and ex-U.S. patent applications that we own, co-own, or have in-licensed from third parties, primarily focused on various aspects of microRNA therapeutics, including various microRNA mimics, and methods of use as microRNA related therapies. Within our patent portfolio, we are the sole owner of multiple U.S. and foreign patent applications related to microRNA therapies, including chemically modified versions of miR-34 not currently used in MRX34 (U.S. Patent No. 8,586,727) and other microRNAs mimics that are possible candidates for future product development as microRNA therapeutics. Further, our patent portfolio includes U.S. 7,960,359 and U.S. 8,563,708, both of which are related to miR-34 and are in-licensed from Asuragen. Specifically, U.S. 7,960,359 is related to use of a miR-34a mimic, for example MRX34, for reducing cell viability of human lung cancer cells, human cancerous T cells, human prostate cancer cells or human skin cancer cells. This patent is expected to expire in 2025. We also are the exclusive licensee with respect to MRX34 of US 9,006,206, which relates to use of miR-34 to treat a cancer associated with p53, and EP2126078, which relates to treatment of certain cancers that are also p53 negative. Both US 9,006,206 and EP 2126078 are co-owned by Rosetta Genomics and Yeda Research & Development. See "Business—Intellectual Property—Our Patent Portfolio" of Part I, Item 1 of our Annual Report for the year ended December 31, 2015 for a more detailed description of the patents we own or license covering our product candidates.

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If the patent applications we hold or have in-licensed with respect to our programs or product candidates fail to issue, if their breadth or strength of protection is threatened, if we abandon or allow owned or in-licensed patents or patent applications that we are responsible for prosecuting to lapse, or if our owned and in-licensed patents and patent applications fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates. We have multiple pending patent applications relating to our product candidates. We cannot offer any assurances about which, if any, patents will issue, the breadth of the claims of any such patent, should it issue, or whether any issued patents will be found invalid and/or unenforceable, will be interpreted narrowly or will be threatened by third parties. 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.

Most of our portfolio is entitled to effective filing dates prior to March 16, 2013. For U.S. patent applications in which patent claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third party, for example a competitor, or instituted by the U.S. Patent and Trademark Office, or USPTO, to determine who was the first to invent any of the subject matter covered by those patent claims. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our participation in an interference proceeding may fail and, even if successful, may result in substantial costs and distract our management.

Further, if we encounter delays in our clinical trials or achieving regulatory approvals, the period of time during which we could market any of our product candidates under patent protection, if approved, would be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we were the first to file any patent application related to our product candidates. Furthermore, an interference proceeding can be provoked by a third party or instituted by the USPTO, to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. In addition, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Even if we obtain patents that cover the manufacture, use and/or sale of our product candidates and such patents are not successfully challenged by any third parties, once the patent life has expired for a product, we may be open to competition, including from generic medications.

If we resume the research and development of our product candidates despite suspending such activities, we may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

Presently we have rights to certain intellectual property through licenses from third parties and under patents that we own or co-own, related to a subset of the known microRNA targets. Because our programs may involve a range of microRNA targets and specific formulations of microRNA mimics directed to such targets, including targets and formulations that may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or otherwise gain the right to use these proprietary rights. We may be unable to acquire or in-license any necessary or desirable third-party intellectual property rights on reasonable terms, or at all. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive now or in the future. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third-party intellectual property rights, including rights related to our lead product candidate, our business, financial condition and prospects for growth could suffer.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that may not be patentable or that we elect not to patent, processes for which patents may be difficult to obtain or enforce, and any other elements of our product candidates' discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary data and processes, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and collaborators, and any other third parties that have access to our proprietary know-how, information or technology, for example, third parties involved in our

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clinical trials. Although we expect all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed, and we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all.

We also seek to preserve the integrity and confidentiality of our data, trade secrets and know-how by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. Moreover, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent material disclosure of the intellectual property related to our technologies to third parties, we will not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, financial condition and results of operations.

If we resume the research and development of our product candidates despite suspending such activities, issued patents covering our product candidates could be found invalid or unenforceable if challenged in court or the USPTO.

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering the manufacture, use or sale, or other aspects of one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation.

Such mechanisms include ex parte re-examination, inter partes review, post grant review and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Similarly, the outcome following administrative review of a patent that we own or license, such as via a reexamination or opposition proceeding before the USPTO or a foreign body, is unpredictable. If a third party were to prevail, we could lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection could have a material adverse impact on our business.

If we resume the research and development of our product candidates despite suspending such activities and are sued for infringing the patent rights or misappropriating the trade secrets of third parties, such litigation could be costly and time consuming.

If we resume the research and development of our product candidates, our commercial success would depend, in part, on our ability to develop, manufacture, market and sell our product candidates and use our proprietary technology without infringing the patent rights of third parties. Numerous third-party U.S. and non-U.S. issued patents and pending applications exist in the area of microRNA. We are aware of certain U.S. and foreign patents and pending patent applications owned by our competitors or other third parties that cover certain miR-34 mimics and therapeutic uses thereof. We are currently monitoring these patents and patent applications. We have and we may in the future pursue available proceedings in the U.S. and foreign patent offices to challenge the validity of these patents and patent applications. In addition, or alternatively, we may consider whether to seek to negotiate a license of rights to technology covered by one or more of such patents and patent applications. If any patents or patent applications cover our product candidates or technologies, we may not be free to manufacture or market

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our product candidates as planned, absent such a license, which may not be available to us on commercially reasonable terms, or at all.

It is also possible that we have failed to identify relevant third-party patents or applications. For example, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our product candidates and technologies because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may be unaware of one or more issued patents that would be infringed by the manufacture, sale or use of a current or future product candidate, or we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our products or the use of our products.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding patent rights with respect to our technology or products candidates, including interferences, oppositions and *inter partes* review proceedings before the USPTO and corresponding foreign patent offices. We also monitor patent prosecution activities and pending applications of competitors and potential competitors in our field in order to identify third party patent rights that could pose a potential threat to our freedom to operate in the market with respect to our product candidates, once commercialized. We are currently pursuing and may in the future pursue available administrative proceedings in the U.S. or foreign patent offices to challenge third party patent rights that could adversely impact our ability to commercialize one or more of our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our current or future product candidates may be subject to claims of infringement of the patent rights of third parties, who may assert infringement claims against us based on existing or future patent rights. Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates and third parties could allege that our technology infringes such claims. Further, because patent applications can take many years to issue, third parties may have currently pending patent applications which may later result in issued patents that our product candidates may infringe, or which such third parties claim are infringed by the use of our technologies. The outcome of patent litigation is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we are sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid, and we may not be able to do this. Proving that a patent is invalid is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on us. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

Although we have suspended our research and development activities, if we resume the development of any of our product candidates and are found to infringe a third party's patent rights, including any patent rights related to miR-34, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate. 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 access to the same technologies licensed to us. 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. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

Parties making claims against us for infringement of their patent rights may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we could be required to redesign our infringing products or obtain a license from such third party to continue developing and commercializing our

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products and technology. However, we may not be able to obtain any required license on commercially reasonable terms, or at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. It may be impossible to redesign our products and technology, or it may require substantial time and monetary expenditure, which could force us to cease commercialization of one or more of our product candidates or some of our business operations, which could materially harm our business. In addition, in any such proceeding, we may be required to pay substantial damages, including treble damages and attorneys' fees in the event we are found liable for willful infringement.

We may be involved in lawsuits or administrative proceedings to protect or enforce our intellectual property rights, which could be expensive, time consuming and unsuccessful.

Competitors may infringe or we may believe that they infringe patents that we own or license. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours is not valid or is unenforceable, or may refuse to stop the other party in such infringement proceeding 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 our patents at risk of being invalidated, held unenforceable or interpreted narrowly, and could put any of our patent applications at risk of not yielding an issued patent. Litigation is uncertain, and we cannot predict whether we would be successful in any such litigation.

Interference proceedings provoked by third parties or brought by the USPTO or any foreign patent authority may be necessary to determine the priority of inventions with respect to our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms, if any license is offered at all. Litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees.

We may not be able to prevent misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the United States. 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. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Legal actions to enforce patent rights or other intellectual property rights that we own or license can be expensive and may involve the diversion of significant management time. In addition, these legal actions could be unsuccessful and could also result in the invalidation of our patents or a finding that they are unenforceable. Moreover, third parties may be able to successfully design around our patents using pre-existing technology, by developing new technology or by using similar technology that is outside the scope of our patents. We may or may not choose to pursue litigation against those that have infringed on our patents, or used them without authorization, due to the associated expense and time commitment of monitoring these activities. If we fail to protect or to enforce our intellectual property rights successfully, our competitive position could suffer, which could harm our results of operations.

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

Filing, prosecuting and defending patents on all of our product candidates throughout the world would be prohibitively expensive, and our patent rights in some countries outside the United States can be less extensive than those in the United States. The requirements for patentability may differ in certain countries, particularly developing countries. For example, unlike other countries, China has a heightened requirement for patentability, and specifically requires a detailed description of medical uses of a claimed therapeutic. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions.

As part of ordinary course prosecution and maintenance activities, we determine whether to seek patent protection outside the United States and in which countries. This also applies to patents we have acquired or in-licensed from third parties. In some cases, this means that we, or our predecessors in interest or licensors of patents within our portfolio, have sought patent protection in a limited number of countries for patents covering our product candidates. Competitors may use our technologies in jurisdictions where we have not pursued and obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as in

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the United States. These products may compete with our products in jurisdictions where we do not have any issued patents and, even in jurisdictions where we have or are able to obtain issued patents, our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing. Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. In addition, certain countries in Europe and certain developing countries, including India and China, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, we may have limited remedies if our patents are infringed or if we are compelled to grant a license to our patents to a third party, which could materially diminish the value of those patents. This could harm the potential value of our product candidates if we decide to resume our research and development activities, which have been suspended.

Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we own or license. Moreover, patent protection must ultimately be sought on a country-by-country basis, which is an expensive and time-consuming process with uncertain outcomes. Accordingly, we may choose not to seek patent protection in certain countries, and we will not have the benefit of patent protection in such countries.

The patent protection and patent prosecution for some of our product candidates may be dependent on our third party licensors.

While we normally seek to obtain the right to control the filing, prosecution, maintenance, defense and enforcement of the patents and patent applications that we in-license relating to our product candidates, there may be times when such activities for patents that relate to our product candidates are controlled by our licensors. For example, we do not have the first right to prosecute, maintain, defend, or enforce the patent rights licensed to us relating to the SMARTICLES technology under our agreement with Marina Biotech, Inc., or Marina. Although we may retain the right to consult on such filing, prosecution, maintenance, defense, and enforcement activities, our overall ability to influence such activities is limited. Moreover, the patent rights we have in-licensed from Marina may be put at risk in litigation or administrative proceedings unrelated to our product candidates. Further, while we seek to have rights to take action to defend our in-licensed patents and patent applications from third-party challenges in the event that our licensors determine not to, we may not be aware of any such potential threats to the intellectual property rights we in-license, or we may be unsuccessful in protecting such intellectual property rights if we respond to any such challenges by third parties.

If these licensors or any of our future licensors fail to appropriately file, prosecute, maintain, defend or enforce our in-licensed patents and patent applications covering any of our product candidates, our ability to develop and commercialize those product candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products.

If we breach any of the agreements under which we license patent rights to use, develop and commercialize our product candidates or our technologies from third parties or, in certain cases, we fail to meet certain development deadlines, we could lose license rights that are important to our business.

We are a party to a number of license agreements under which we are granted rights to intellectual property that are important to our business and we expect that we may need to enter into additional license agreements in the future. These include our exclusive cross-license agreement with Asuragen, our exclusive licenses from Yale University, or Yale, Marina, the University of Zurich, and Rosetta Genomics Ltd., or Rosetta Genomics.

Our existing license agreements, except our cross-license agreement with Asuragen, generally impose, and we expect that future license agreements will impose on us, various development, regulatory and/or commercial diligence obligations, and financial obligations, such as payment of milestones and/or royalties. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we may not be able to market products covered by the license. Our business could suffer, for example, if any current or future licenses terminate, if the licensors fail to abide by the terms of the license, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. See “Business — Strategic

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Partnerships and Licenses” of Part I, Item 1 of our Annual Report for the year ended December 31, 2015 for a description of our license agreements, which sets forth the material terms and obligations, including a description of the termination provisions, under our agreements with Asuragen, Yale, Marina, the University of Zurich and Rosetta Genomics.

As we have done previously, if we resume the research and development of our product candidates, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we cannot provide any assurances that third-party patents do not exist that might be enforced against our current product candidates or future products in the absence of such a license. We may fail to obtain any of these licenses on commercially reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property that we have licensed arise, we would expect to exercise all rights and remedies available to us, including seeking to cure any breach by us, and otherwise seek to preserve our rights under the patents licensed to us. However, we may not be able to do so in a timely manner, at an acceptable cost or at all. Generally, the loss of any one of our current licenses, or any other license we may acquire in the future, could prevent or impair our ability to successfully develop and commercialize the affected product candidates and thus materially harm our business, prospects, financial condition and results of operations.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

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, provide a barrier to entry against our competitors or potential competitors, or permit us to maintain our competitive advantage. Moreover, if a third party has intellectual property rights that cover the practice of our technology, we may not be able to fully exercise or extract value from our intellectual property rights. The following examples are illustrative:

- others may be able to make compounds that are similar to our product candidates but that are not covered by the claims of the patents that we own or license;
- we or our licensors or collaborators might not have been the first to make the inventions covered by an issued patent or pending patent application that we own or license;
- we or our licensors or collaborators might not have been the first to file patent applications covering an invention;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing or misappropriating our intellectual property rights;
- pending patent applications that we own or license may not lead to issued patents;

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- issued patents that we own or license may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop or in-license additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

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

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

We were previously involved in discussions with Yale regarding the inventorship and ownership of certain patents and patent applications licensed to us by Asuragen. An independent third party expert was engaged to determine the inventorship and the ownership of patents and patent applications potentially subject to Yale and Asuragen co-ownership. This determination confirmed Asuragen's sole ownership of the patents and patent applications where co-ownership had been under consideration and resulted in a determination that Yale should be removed as a co-owner of one of the pending patent applications, an action we are currently undertaking.

Although we seek to protect our ownership of our patents and other intellectual property by ensuring that our agreements with our employees and certain collaborators and other third parties with whom we do business include provisions requiring, for instance, such parties to assign rights in inventions to us, we may be subject to claims that former or current employees, collaborators or other third parties have an ownership interest in our patents, in-licensed patents or other intellectual property. In some situations, our confidentiality agreements may conflict with, or be subject to, the rights of third parties with whom our employees, consultants or advisors have previous employment or consulting relationships, and further, many of our consultants are currently retained by other biotechnology or pharmaceutical companies, including our competitors or potential competitors, and may be subject to conflicting obligations to these third parties. To the extent that our employees, consultants or contractors use any intellectual property owned by third parties in their work for us, disputes may arise as to the ownership of rights in any related or resulting know-how and inventions, arising, for example, from such conflicting obligations of consultants, employees or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property.

Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Obtaining and maintaining 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.

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent prosecution process. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or patent applications will be due to be paid to the USPTO and various patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ reputable law firms and other professionals and rely on such third parties to effect payment of these fees with respect to the USPTO and non-U.S. patent agencies with respect to the patents and patent applications we own, and we rely upon our licensors to effect payment of these fees with respect to the patents and patent applications that we in-license. Even if we do not control prosecution and maintenance of our in-licensed patents, we may be responsible for reimbursing our licensors for some or all of the costs associated with such activities. If we fail to make timely payment to our licensors for such fees, our licensors may have the right to terminate the affected license, in which event we would not be able to market products covered by the license. We also employ reputable law firms and other professionals to help us comply with the various documentary and other procedural requirements with respect to the patents and patent applications that we own. In some cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of

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patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

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

As is the case with other biotechnology companies, our success is heavily dependent on patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. Some of our patent claims may be affected by the recent U.S. Supreme Court decision in *Association for Molecular Pathology v. Myriad Genetics*. In *Myriad*, the Supreme Court held that unmodified isolated fragments of genomic sequences, such as the DNA constituting the BRCA1 and BRCA2 genes, are not eligible for patent protection because they constitute a product of nature. The exact boundaries of the Supreme Court's decision remain unclear as the Supreme Court did not address other types of nucleic acids, such as isolated microRNAs. Nevertheless, our patent portfolio contains claims of various types and scope, including chemically modified mimics, such as in MRX34, as well as methods of medical treatment. In our view, the presence of varying claims in our patent portfolio significantly reduces, but does not eliminate, our exposure to potential validity challenges under *Myriad* or future judicial decisions. However, it is not yet clear what, if any, impact this recent Supreme Court decision or future decisions will have on the operation of our business.

For our U.S. patent applications containing a claim not entitled to priority before March 16, 2013, there is a greater level of uncertainty in the patent law. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The USPTO has promulgated regulations and developed 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, did not come into effect until March 16, 2013. Accordingly, it is not yet 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.

An important change introduced by the Leahy-Smith Act is that, as of March 16, 2013, the United States transitioned to a "first-to-file" system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. A third party that files a patent application in the USPTO after that date but before us could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by the third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Furthermore, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our technology and the prior art allow our technology to be patentable over the prior art. Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we were the first to either (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our patents or patent applications.

Among some of the other changes introduced by the Leahy-Smith Act are changes that limit where a patentee may file a patent infringement suit and providing opportunities for third parties to challenge any issued patent in the USPTO. This applies to all of our U.S. patents, even those issued before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal court necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action.

We may be subject to claims that our employees or consultants or independent contractors have wrongfully used or disclosed confidential information or trade secrets of third parties or that our employees or consultants have wrongfully used or disclosed alleged trade secrets of former or other employers.

Many of our employees, independent contractors and consultants, including our senior management, have been previously employed or retained by other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of third parties in their work for us, and do not perform work for us that is in conflict with their obligations to another employer or any other entity, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise improperly used or disclosed confidential information, including trade secrets or other proprietary information, of a former employer or other third parties. We may also be subject to claims that an employee, advisor, consultant, or independent contractor performed work for us that conflicts with that person's obligations to a third party, such as an employer, and thus, that the third party has an ownership interest in the intellectual property arising out of work performed for us. We are not aware of any threatened or pending claims related to these matters, but in the future litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable personnel or intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property disputes could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

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

Risks Related to Our Common Stock

Our stock price is volatile and our stockholders may not be able to resell shares of our common stock at or above the price they paid.

The trading price of our common stock is highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. These factors include those discussed in this "Risk Factors" section of this report and others such as:

- announcement of a strategic transaction, including the acquisition of our company or its assets;
- announcements relating to collaborations that we may enter into with respect to the development or commercialization of our product candidates;
- announcements relating to the receipt, modification or termination of government contracts or grants;
- success of our competitors in discovering, developing or commercializing products;
- product liability claims related to our clinical trials or product candidates;
- prevailing economic conditions;
- additions or departures of key personnel;
- business disruptions caused by earthquakes or other natural disasters;
- disputes concerning our intellectual property or other proprietary rights;
- FDA or other U.S. or foreign regulatory actions affecting us or our industry;
- sales of our common stock by us, our executive officers and directors or our stockholders in the future;

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- healthcare reform measures in the United States and outside the United States;
- future sales or issuances of equity or debt securities by us;
- lack of an active, liquid and orderly market in our common stock;
- fluctuations in our quarterly operating results; and
- the issuance of new or changed securities analysts' reports or recommendations regarding us.

In addition, the stock markets in general, and the markets for pharmaceutical, biopharmaceutical and biotechnology stocks in particular, have experienced extreme volatility that have been often unrelated to the operating performance of the issuer. These broad market fluctuations may adversely affect the trading price or liquidity of our common stock. In the past, when the market price of a stock has been volatile, holders of that stock have sometimes instituted securities class action litigation against the issuer. If any of our stockholders were to bring such a lawsuit against us, we could incur substantial costs defending the lawsuit and the attention of our management would be diverted from the operation of our business.

Our common stock may be delisted from the NASDAQ Global Market if we are unable to maintain compliance with NASDAQ's continued listing standards.

NASDAQ imposes, among other requirements, continued listing standards including minimum bid and public float requirements. The price of our common stock must trade at or above \$1.00 to comply with NASDAQ's minimum bid requirement for continued listing on the NASDAQ. If our stock trades at bid prices of less than \$1.00 for a period in excess of 30 consecutive business days, the NASDAQ could send a deficiency notice to us for not remaining in compliance with the minimum bid listing standards. During the third quarter of fiscal year 2016, our common stock never traded below \$1.00. However, if the closing bid price of our common stock fails to meet NASDAQ's minimum closing bid price requirement, or if we otherwise fail to meet any other applicable requirements of the NASDAQ and we are unable to regain compliance, NASDAQ may make a determination to delist our common stock.

Any delisting of our common stock could adversely affect the market liquidity of our common stock and the market price of our common stock could decrease. Furthermore, if our common stock were delisted it could adversely affect our ability to obtain financing for the continuation of our operations and/or result in the loss of confidence by investors, customers, suppliers and employees

Our principal stockholders and management own a significant percentage of our stock and are able to exert significant control over matters subject to stockholder approval.

Based on the beneficial ownership of our common stock as of September 30, 2016, our officers and directors, together with holders of 5% or more of our outstanding common stock and their respective affiliates, beneficially own approximately 74.6% of our common stock. Accordingly, these stockholders have significant influence over the outcome of corporate actions requiring stockholder approval, including the election of directors, any merger, consolidation or sale of all or substantially all of our assets or any other significant corporate transaction. The interests of these stockholders may not be the same as or may even conflict with your interests. For example, these stockholders could delay or prevent a change of control of our company, even if such a change of control would benefit our other stockholders, which could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of our company or our assets and might affect the prevailing market price of our common stock. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise.

We are an "emerging growth company" and we cannot be certain if the reduced disclosure requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act, and may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies" including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

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In addition, Section 102 of the JOBS Act also provides that an “emerging growth company” can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, or the Securities Act, for complying with new or revised accounting standards. An “emerging growth company” can therefore delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. However, we are choosing to “opt out” of such extended transition period, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision to opt out of the extended transition period for complying with new or revised accounting standards is irrevocable.

Future sales of our common stock or securities convertible or exchangeable for our common stock may depress our stock price.

If our existing stockholders or holders of our options sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the trading price of our common stock could decline. The perception in the market that these sales may occur could also cause the trading price of our common stock to decline. As of September 30, 2016, we have a total of 20,835,868 shares of common stock outstanding.

In addition, based on the number of shares subject to outstanding awards under our 2008 Long Term Incentive Plan, or 2008 Stock Plan, as of September 30, 2016, and including the initial reserves under our 2015 Equity Incentive Award Plan, or 2015 Plan, and Employee Stock Purchase Plan, or ESPP, approximately 3.9 million shares of common stock that are either subject to outstanding options, outstanding but subject to vesting, or reserved for future issuance under the 2008 Stock Plan, 2015 Plan or ESPP will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules. We also filed a registration statement permitting certain shares of common stock issued in the future pursuant to the 2008 Plan, 2015 Plan and ESPP to be freely resold by plan participants in the public market, subject to applicable vesting schedules and, for shares held by directors, executive officers and other affiliates, volume limitations under Rule 144 under the Securities Act. The 2015 Plan and ESPP also contain provisions for the annual increase of the number of shares reserved for issuance under such plans, which shares we also intend to register. If the shares we may issue from time to time under the 2008 Stock Plan, 2015 Plan or ESPP are sold, or if it is perceived that they will be sold, by the award recipient in the public market, the trading price of our common stock could decline.

Certain holders of approximately 13.9 million shares of our common stock at September 30, 2016 are entitled to rights with respect to the registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares purchased by affiliates. Sales of such shares could also cause the trading price of our common stock to decline.

An active, liquid and orderly market for shares of our common stock may not be sustained.

Prior to our initial public offering in October 2015, there had been no public market for our common stock, and an active public market for our shares may not be sustained. Further, certain of our existing institutional investors, including investors affiliated with certain of our directors, purchased approximately 2.4 million shares of common stock in our initial public offering and consequently fewer shares may be actively traded in the public market because these stockholders are restricted from selling the shares by restrictions under applicable securities laws, which would reduce the liquidity of the market for our common stock. If an active market for shares of our common stock is not maintained it may be difficult for our stockholders to sell their shares at the time they wish to sell them or at a price that they consider reasonable or it may result in volatility in our stock price. An inactive market may also impair our ability to raise capital by selling shares and may impair our ability to acquire other businesses or technologies or in-license new product candidates using our shares as consideration.

Our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of our operating expenses;
- receipt, modification or termination of government contracts or grants, and the timing of payments we receive under these arrangements;

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- our execution of any collaborative, licensing or similar arrangements, and the timing of payments we may make or receive under these arrangements; and
- any intellectual property infringement lawsuit or opposition, interference or cancellation proceeding in which we may become involved.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

Provisions of our charter documents or Delaware law could delay or prevent an acquisition of our company, even if the acquisition would be beneficial to our stockholders, and could make it more difficult for you to change management.

Provisions in our amended and restated certificate of incorporation and our amended and restated bylaws may discourage, delay or prevent a merger, acquisition or other change in control that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares. In addition, these provisions may frustrate or prevent any attempt by our stockholders to replace or remove our current management by making it more difficult to replace or remove our board of directors. These provisions include:

- a classified board of directors so that not all directors are elected at one time;
- a prohibition on stockholder action through written consent;
- no cumulative voting in the election of directors;
- the exclusive right of our board of directors to elect a director to fill a vacancy created by the expansion of the board of directors or the resignation, death or removal of a director;
- a requirement that special meetings of stockholders be called only by the board of directors, the chairman of the board of directors, the chief executive officer or, in the absence of a chief executive officer, the president;
- an advance notice requirement for stockholder proposals and nominations;
- the authority of our board of directors to issue preferred stock with such terms as our board of directors may determine; and
- a requirement of approval of not less than 66 2/3% of all outstanding shares of our capital stock entitled to vote to amend any bylaws by stockholder action, or to amend specific provisions of our certificate of incorporation.

In addition, Delaware law prohibits a publicly held Delaware corporation from engaging in a business combination with an interested stockholder, generally a person who, together with its affiliates, owns or within the last three years has owned 15% or more of our voting stock, for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in a prescribed manner. Accordingly, Delaware law may discourage, delay or prevent a change in control of our company. Furthermore, our amended and restated certificate of incorporation will specify that the Court of Chancery of the State of Delaware will be the sole and exclusive forum for most legal actions involving actions brought against us by stockholders. We believe this provision benefits us by providing increased consistency in the application of Delaware law by chancellors particularly experienced in resolving corporate disputes, efficient administration of cases on a more expedited schedule relative to other forums and protection against the burdens of multi-forum litigation. However, the provision may have the effect of discouraging lawsuits against our directors and officers. The enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that, in connection with any applicable action brought against us, a court could find the choice of forum provisions contained in our amended and restated certificate of incorporation to be inapplicable or unenforceable in such action.

Provisions in our charter and other provisions of Delaware law could limit the price that investors are willing to pay in the future for shares of our common stock.

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Our employment agreements with our officers may require us to pay severance benefits to any of those persons who are terminated in connection with a change of control of us, which could harm our business, financial condition or results of operations.

Our officers are parties to employment agreements providing for aggregate cash payments of up to approximately \$2.4 million at September 30, 2016 for severance and other benefits in the event of a termination of employment in connection with a change of control of us. The payment of these severance benefits could harm our business, financial condition and results of operations. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

We do not anticipate paying any cash dividends on our common stock in the foreseeable future; therefore, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

We have never declared or paid cash dividends on our common stock. We do not anticipate paying any cash dividends on our common stock in the foreseeable future. We currently intend to retain all available funds and any future earnings to fund the development and growth of our business. In addition, the terms of any future debt financing arrangement may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

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

The trading market for our common stock will depend, in part, on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no securities or industry analysts commence coverage of our company, the trading price for our common stock would likely be negatively impacted. In the event securities or industry analysts initiate coverage, if one or more of the analysts who cover us downgrade our common stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. In addition, if our operating results fail to meet the forecast of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our common stock could decrease, which might cause our stock price and trading volume to decline.

Changes in, or interpretations of, accounting rules and regulations could result in unfavorable accounting charges or require us to change our compensation policies.

Accounting methods and policies for biopharmaceutical companies, including policies governing revenue recognition, research and development and related expenses and accounting for stock-based compensation, are subject to further review, interpretation and guidance from relevant accounting authorities, including the SEC. Changes to, or interpretations of, accounting methods or policies may require us to reclassify, restate or otherwise change or revise our financial statements, including those contained in this periodic report.

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

Unregistered Sales of Equity Securities

None.

Use of Proceeds

On September 30, 2015, the U.S. Securities and Exchange Commission declared effective our registration statement on Form S-1 (File No. 333-206544), as amended, filed in connection with our initial public offering. Pursuant to the registration statement, we registered the offer and sale of 6,250,000 shares of our common stock with an aggregate offering price of approximately \$43.8 million, as well as the issuance of an additional 704,962 shares of our common stock pursuant to the underwriters' partial exercise of their option to purchase additional shares, for an aggregate offering price of approximately \$4.9 million. In total, we issued and sold an aggregate of 6,954,962 shares of our common stock at a price to the public of \$7.00 per share for an aggregate offering price of approximately \$48.7 million. The managing underwriters of the offering were Citigroup, Leerink Partners, Oppenheimer & Co. and Cantor Fitzgerald & Co. After deducting underwriting discounts and commissions and offering expenses paid or payable by us of \$5.0 million, the aggregate net proceeds from the offering were

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\$43.7 million. No offering expenses were paid or are payable, directly or indirectly, to our directors or officers, to persons owning 10% or more of any class of our equity securities or to any of our affiliates.

The net proceeds from our initial public offering have been invested in a variety of capital preservation investments, including short-term, investment-grade, interest-bearing instruments and U.S. government securities. Following the close of the Company's Phase 1 clinical trial of MRX34, the Company is evaluating strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company, and has suspended further research and development activities to reduce operating expenses while it evaluates these opportunities. We currently expect to primarily use the remaining net proceeds from our initial public offering for working capital and other general corporate purposes, which include our activities to evaluate and pursue strategic alternatives.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

The information set forth below is included herein for the purpose of providing the disclosure required under "Item 2.05 - Costs Associated with Exit or Disposal Activities" of Form 8-K.

On November 7, 2016, the Company initiated activities to implement a workforce reduction to reduce operating expenses and streamline its operations following the Company's decision to close its Phase 1 study of MRX34 in September 2016. The Company intends to reduce the total number of full-time employees from 36 to 12. The Company expects the majority of the employees included in the reduction will separate from the Company during the fourth quarter of 2016 and expects the remainder will discontinue employment with the Company during the first quarter of 2017. In addition, the Company has entered into retention agreements with certain employees necessary to adequately close the Phase 1 trial of MRX34 and maintain the operations of the Company. The Company estimates that it will incur aggregate cash expenses of approximately \$1.6 million under the workforce reduction, of which \$1.3 million is attributed to one-time severance and benefits payments and \$0.3 million relates to retention payments.

The information set forth below is included herein for the purpose of providing the disclosure required under "Item 5.02 - Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officers" of Form 8-K.

On November 8, 2016, Clay B. Siegall, Ph.D. notified the Company's board of directors (the "Board") that he will resign from the Board, effective as of December 31, 2016.

On November 10, 2016, Jon Irvin, our Vice President of Finance and principal accounting officer was included in the Company's workforce reduction described above. His last day as an employee of the Company is December 2, 2016.

Item 6. Exhibits

| Exhibit Number | Description of Document | Incorporated by Reference | | | Provided Herewith |
|----------------|---|---------------------------|-----------|--------|-------------------|
| | | Form | Date | Number | |
| 3.1 | Amended and Restated Certificate of Incorporation. | 8-K | 10/6/2015 | 3.1 | |
| 3.2 | Amended and Restated Bylaws. | 8-K | 10/6/2015 | 3.2 | |
| 4.1 | Reference is made to Exhibits 3.1 through 3.2. | | | | |
| 4.2 | Form of Common Stock Certificate. | S-1/A | 9/18/2015 | 4.2 | |
| 31.1 | Certification of Chief Executive Officer required by Rule 13a-14(a) or Rule 15d-14(a). | | | | X |
| 31.2 | Certification of Chief Financial Officer required by Rule 13a-14(a) or Rule 15d-14(a). | | | | X |
| 32.1* | Certification required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350) | | | | X |
| 101.INS | XBRL Instance Document | | | | X |
| 101.SCH | XBRL Taxonomy Extension Schema Document | | | | X |
| 101.CAL | XBRL Taxonomy Extension Calculation Linkbase Document | | | | X |
| 101.DEF | XBRL Taxonomy Extension Definition Linkbase Document | | | | X |
| 101.LAB | XBRL Taxonomy Extension Label Linkbase Document | | | | X |
| 101.PRE | XBRL Taxonomy Extension Presentation Linkbase Document | | | | X |

* The certification attached as Exhibit 32.1 that accompanies this Quarterly Report on Form 10-Q is not deemed filed with the SEC and is not to be incorporated by reference into any filing of Mirra Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Quarterly Report on Form 10-Q, irrespective of any general incorporation language contained in such filing.

SIGNATURES

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

MIRNA THERAPEUTICS, INC.

(Registrant)

Date: November 10, 2016

/s/ Paul Lammers

Paul Lammers, M.D., M.Sc.
Chief Executive Officer
(Principal Executive Officer)

Date: November 10, 2016

/s/ Alan Fuhrman

Alan Fuhrman
Chief Financial Officer
(Principal Financial Officer)

CERTIFICATION OF THE CHIEF EXECUTIVE OFFICER
PURSUANT TO
SECURITIES EXCHANGE ACT RULES 13A-14(A) AND 15D-14(A)

I, Paul Lammers, certify that:

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

Date: November 10, 2016

/s/ PAUL LAMMERS

Paul Lammers, M.D., M.Sc.

Chief Executive Officer

(Principal Executive Officer)

CERTIFICATION OF THE CHIEF FINANCIAL OFFICER
PURSUANT TO
SECURITIES EXCHANGE ACT RULES 13A-14(A) AND 15D-14(A)

I, Alan Fuhrman, certify that:

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

Date: November 10, 2016

/s/ ALAN FUHRMAN

Alan Fuhrman

Chief Financial Officer

(Principal Financial Officer)

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

In connection with the Quarterly Report of Mima Therapeutics, Inc. (the "Company") on Form 10-Q for the fiscal quarter ended September 30, 2016, as filed with the Securities and Exchange Commission (the "Report"), Paul Lammers, Chief Executive Officer of the Company, and Alan Fuhrman, Chief Financial Officer of the Company, respectively, do each hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

- The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- The information in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 10, 2016

/s/ PAUL LAMMERS

Paul Lammers, M.D., M.Sc.

Chief Executive Officer

(Principal Executive Officer)

/s/ ALAN FUHRMAN

Alan Fuhrman

Chief Financial Officer

(Principal Financial Officer)