

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

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**FORM 10-Q**

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(Mark One)

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

For the quarterly period ended March 31, 2017  
or

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

Commission File Number: 001-37566

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

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

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

**1250 South Capital of Texas Highway**  
Austin, TX (Address of principal executive offices)

**78746**  
(Zip Code)

**(512) 901-0950**  
(Registrant's telephone number, including area code)

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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, a smaller reporting company, or an emerging growth company. See definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/> (Do not check if a smaller reporting company)	Smaller reporting company	<input type="checkbox"/>
Emerging growth company	<input checked="" type="checkbox"/>		

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

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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 May 2, 2017 there were 20,856,693 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)

	March 31, 2017 (unaudited)	December 31, 2016
<b>Assets</b>		
Current Assets:		
Cash and cash equivalents	\$ 17,121	\$ 16,432
Short-term marketable securities	40,408	44,066
Prepaid expenses and other current assets	620	882
Total current assets	58,149	61,380
Property and equipment, net	26	354
Restricted cash	2,433	2,432
Total assets	<u>\$ 60,608</u>	<u>\$ 64,166</u>
<b>Liabilities and Stockholders' Equity (Deficit)</b>		
Current Liabilities:		
Accounts payable	\$ 371	\$ 361
Accrued expenses	4,486	2,400
Total current liabilities	4,857	2,761
Lease obligations, long-term	—	1,053
Total liabilities	4,857	3,814
Stockholders' Equity (Deficit):		
Preferred stock, \$0.001 par value, 5,000,000 shares authorized at March 31, 2017 and December 31, 2016; 0 shares outstanding at March 31, 2017 and December 31, 2016	—	—
Common stock, \$0.001 par value; 250,000,000 shares authorized at March 31, 2017 and December 31, 2016; 20,856,693 and 20,841,393 shares issued and outstanding at March 31, 2017 and December 31, 2016, respectively	21	21
Additional paid in capital	163,518	163,126
Accumulated deficit	(107,771)	(102,791)
Other comprehensive loss	(17)	(4)
Total stockholders' equity	55,751	60,352
Total liabilities and stockholders' equity	<u>\$ 60,608</u>	<u>\$ 64,166</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 March 31,	
	2017	2016
Operating expenses:		
Research and development	\$ 242	\$ 4,523
General and administrative	2,264	2,130
Restructuring charges	2,557	—
Total operating expenses	5,063	6,653
Other income:		
Interest income	86	82
Net loss attributable to common stockholders	<u>\$ (4,977)</u>	<u>\$ (6,571)</u>
Other comprehensive loss:		
Unrealized gain/ (loss) on available for sale securities, net of tax	(13)	9
Total other comprehensive loss	(4,990)	(6,562)
Net loss per share attributable to common stockholders—basic and diluted	<u>\$ (0.24)</u>	<u>\$ (0.32)</u>
Common shares used to compute basic and diluted net loss per share attributable to common stockholders	20,850,494	20,830,555

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

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

(in thousands)

	Three Months Ended March 31,	
	2017	2016
<b>Operating activities</b>		
Net loss	\$ (4,977)	\$ (6,571)
Adjustment to reconcile net loss to net cash used in operating activities:		
Restructuring charges	2,557	—
Depreciation and amortization	5	19
Stock-based compensation	364	447
Net amortization of premium/ discounts on marketable securities	87	19
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	262	(192)
Accounts payable	10	(2,448)
Accrued expenses	(1,524)	(247)
Net cash used in operating activities	(3,216)	(8,973)
<b>Investing activities</b>		
Purchases of marketable securities	(12,019)	(27,722)
Maturities of marketable securities	15,577	—
Proceeds from the sale of equipment	325	—
Purchases of property and equipment	(3)	(143)
Net cash provided by/ (used in) investing activities	3,880	(27,865)
<b>Financing activities</b>		
Proceeds from the exercise of stock options	25	—
Cash provided by financing activities	25	—
Net increase (decrease) in cash and cash equivalents	689	(36,838)
Cash and cash equivalents at beginning of period	16,432	89,713
Cash and cash equivalents at end of period	\$ 17,121	\$ 52,875

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 that has historically 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 began to evaluate its strategic alternatives focusing on enhancing stockholder value, including the possibility of a merger or sale of the Company. Mirna has discontinued further research and development activities (see Note 9) to reduce operating expenses while it evaluates these opportunities. The Company is located in Austin, Texas.

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 \$57.5 million at March 31, 2017 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 March 31, 2017 and for the three months ended March 31, 2017 and 2016, 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 March 31, 2017, and the results of its operations and cash flows for the interim periods ended March 31, 2017 and 2016. Such adjustments are of a normal and recurring nature. The interim financial data as of March 31, 2017 is not necessarily indicative of the results to be expected for the year ending December 31, 2017, 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, 2016 included in the Company’s Form 10-K, most recently filed with the Securities and Exchange Commission on March 15, 2017.

**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. Amounts included in the financial statements, such those recorded in restructuring charges, can result from a complex series of judgments about future events and uncertainties and can heavily rely on estimates and assumptions. Actual results could differ from those estimates.

*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***

Prior to the discontinuation of the Company's research and development activities, the Company estimated pre-clinical study and clinical trial expenses pursuant to contracts with research institutions and contract research organizations that conducted and managed pre-clinical studies and clinical trials on the Company's behalf. These estimates were based on the level of service performed and the underlying agreement. Further, the Company accrued expenses related to clinical trials based on the level of patient enrollment and other activities according to the related agreements. The Company monitored patient enrollment levels and other activities to the extent reasonably possible and adjusted estimates accordingly. If actual costs incurred or the timing of services varied from the Company's estimate, the Company adjusted the accrual accordingly. On September 20, 2016, the Company announced its decision to close the ongoing Phase 1 study of MRX34 and halted enrollment and dosing of patients in the study.

### ***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 equal to the grant date fair value of stock options on a straight-line basis over the requisite service period less actual forfeitures.

### ***Restructuring charges***

Following the closing of the Phase 1 MRX34 clinical trial, the Company implemented a workforce reduction in the fourth quarter of 2016 to reduce operating expenses while it evaluates strategic alternatives. The majority of severance and benefits payments were settled during the first quarter of 2017. The Company entered into retention agreements with key employees necessary to close the Phase 1 clinical trial of MRX34 and maintain the continued operations of the Company. Under the retention agreements, employees must remain with the Company until June 30, 2017 or until terminated by the Company without cause prior to such date. The Company has recognized the restructuring liability for such retention agreements over the employees' service period.

In accordance with ASC 420, *Exit and Disposal Cost Obligations*, the Company has also recognized contract termination costs in connection with a leased property it intended to occupy as its corporate headquarters and research facility, as well as a temporary lab in use prior to the discontinuation of the Company's research and development activities. In addition, the Company has recognized asset impairments related to its lab equipment used in the Phase 1 clinical trial, construction in process, and other property and equipment for which the Company does not expect to receive a future benefit.

### ***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 March 31, 2017 and December 31, 2016, 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 months ended March 31, 2017 or 2016.

### ***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 for the Company's lease of approximately 23,578 square feet of office and laboratory space in the event of default. The restricted cash consists of cash providing security under the terms of the lease described in Note 10.

As a result of the Lease Termination Agreement and Release described in Note 12, the landlord will release the letter of credit described above to the Company and the balance of restricted cash will be reclassified to cash and cash equivalents.

### ***Marketable Securities***

Marketable securities with maturities at purchase beyond one year, but less than twenty-four months, may be classified as short-term marketable securities based on their highly liquid nature and because such marketable securities represent the investment of cash that is available for current operations. Marketable securities with maturities at purchase beyond twenty-four months 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.” If the decline is other-than-temporary, the investment is marked to market through a charge to the Company’s statement of operations and comprehensive loss.

### ***Recently Issued and Adopted 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. We adopted ASU 2016-09 in the quarter ended March 31, 2017 with an effective date of January 1, 2017 and made a policy election to account for forfeitures as they occur. In addition, the Company increased both its net operating loss deferred tax asset and its valuation allowance upon adoption of ASU 2016-09. This did not have an impact on net stockholders' equity as the incremental deferred tax assets were fully offset by a corresponding increase in the deferred tax asset valuation allowance as of March 31, 2017. The cumulative effect of adoption was recorded in the quarter ended March 31, 2017 and did not have a material impact on our condensed financial statements.

### 3. Marketable Securities

The following table summarizes the available-for-sale securities held at March 31, 2017 and December 31, 2016 (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
<b>March 31, 2017</b>				
U.S. government agency securities and treasuries	\$ 39,599	\$ —	\$ (17)	\$ 39,582
Corporate debt securities	826	—	—	826
Total available-for-sale securities	<u>\$ 40,425</u>	<u>\$ —</u>	<u>\$ (17)</u>	<u>\$ 40,408</u>
<b>December 31, 2016</b>				
U.S. government agency securities and treasuries	\$ 42,516	\$ 4	\$ (8)	\$ 42,512
Corporate debt securities	1,554	—	—	1,554
Total available-for-sale securities	<u>\$ 44,070</u>	<u>\$ 4</u>	<u>\$ (8)</u>	<u>\$ 44,066</u>

There were no available-for-sale securities held as of March 31, 2017 that 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 March 31, 2017 and December 31, 2016 (in thousands):

	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
<b>March 31, 2017</b>				
Assets:				
Cash and cash equivalents				
Cash	\$ 2,399	\$ 2,399	\$ —	\$ —
Money market funds	14,722	14,722	—	—
Total cash and cash equivalents	17,121	17,121	—	—
Marketable securities				
U.S. government agency securities and treasuries	39,582	—	39,582	—
Corporate debt securities	826	—	826	—
Total marketable securities	40,408	—	40,408	—
Restricted cash	2,433	2,433	—	—
Total assets	<u>\$ 59,962</u>	<u>\$ 19,554</u>	<u>\$ 40,408</u>	<u>\$ —</u>

	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
<b>December 31, 2016</b>				
Assets:				
Cash and cash equivalents				
Cash	\$ 2,785	\$ 2,785	\$ —	\$ —
Money market funds	\$ 9,647	\$ 9,647	\$ —	\$ —
US government agency securities and treasuries	\$ 4,000	—	4,000	—
Total cash and cash equivalents	16,432	12,432	4,000	—
Marketable securities				
U.S. government agency securities and treasuries	42,512	—	42,512	—
Corporate debt securities	1,554	—	1,554	—
Total marketable securities	44,066	—	44,066	—
Restricted cash	2,432	2,432	—	—
Total assets	\$ 62,930	\$ 14,864	\$ 48,066	\$ —

#### *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 March 31, 2017 and December 31, 2016, cash and cash equivalents are comprised of cash, money market accounts, and U.S. government agency securities and treasuries.

#### *Marketable securities*

The cost of available-for-sale securities is adjusted for amortization of premiums and accretion of discounts to maturity. At March 31, 2017 and December 31, 2016, 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 March 31, 2017, available for sale securities of approximately \$40.0 million were in an unrealized loss position of \$17,000. 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 March 31, 2017 and December 31, 2016.

## 5. Property and Equipment

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

	March 31, 2017	December 31, 2016
Furniture, computers and equipment	\$ 62	\$ 385
Accumulated depreciation	(36)	(31)
	\$ 26	354

Depreciation expense was approximately \$5,000 and \$19,000 for the three months ended March 31, 2017 and 2016, respectively.

In February 2017, the Company entered into an Asset Purchase Agreement for the sale of certain of the Company's lab equipment ("Lab Equipment") with a third party for cash consideration of \$325,000. The selling price of the Lab Equipment approximated its book value at December 31, 2016.

## 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 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 462,934 options outstanding in the 2008 Plan at March 31, 2017 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. As of March 31, 2017, 316,932 shares have been transferred from the 2008 Long Term Incentive Plan to the 2015 Equity Incentive Plan for awards that have terminated, expired, or lapsed. Under the 2015 Plan, 1,671,800 shares of the Company's common stock were initially authorized and reserved for issuance. In addition, 1,041,526 shares of the Company's common stock were authorized and reserved for issuance in the first quarter of 2017, for a total of 3,030,258 authorized for grant under the 2015 Plan at March 31, 2017.

### *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. Shares available for future purchase under the ESPP were 369,960 at March 31, 2017; however, as of December 2016, the Company has suspended future issuances of the Company's common stock under the ESPP plan.

#### Stock Option Activity

The Company's stock option activity for the three months ended March 31, 2017 was as follows:

	Number of Shares	Weighted- Average Exercise Price	Weighted-Average Contractual Life (years)
Outstanding at December 31, 2016	1,905,214	\$ 5.39	7.49
Granted	—	—	
Exercised	(15,300)	1.65	
Forfeited/canceled	(278,593)	5.52	
Outstanding at March 31, 2017	1,611,321	\$ 5.40	8.12
Options exercisable at March 31, 2017	692,798	\$ 5.45	7.30

#### Stock Compensation Expense

Total stock-based compensation expense for the three and three months ended March 31, 2017 was recognized as follows in the statements of comprehensive loss (in thousands):

	Three Months Ended March 31,	
	2017	2016
Research and development expense	\$ 41	\$ 171
General and administrative expense	323	276
Total stock based compensation	\$ 364	\$ 447

As of March 31, 2017, there was approximately \$2.9 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 2.4 years. There were no restricted stock units or stock appreciation rights granted under the 2015 Plan as of March 31, 2017.

## 8. Income Taxes

The Company has not recorded a provision for income taxes as of March 31, 2017 due to reported net losses since inception.

During the three months ended March 31, 2017 and 2016, 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. Restructuring Charges

On September 20, 2016, Mirna announced its decision to close the ongoing Phase 1 study of MRX34 and voluntarily halted the enrollment and dosing of patients in the study. Following the announcement, the Company received verbal notice from the U.S. Food and Drug Administration ("FDA") on September 28, 2016 that its Investigational New Drug MRX34 had been placed on full clinical hold. Following the Company's announcement and notification from the FDA, the Company's board of directors approved a reduction of the total number of full-time employees from 36 to 12. As of March 31, 2017, the total number of full-time employees was 7. The Company also committed to retention payments to certain key employees if such employees remained with the Company until June 30, 2017 or were terminated by Mirna without cause prior to such date. Total estimated restructuring charges are approximately \$7.1 million and are expected to be incurred through June 30, 2017. Cumulative restructuring charges of \$7.0 million have been recognized as of March 31, 2017, of which \$4.4 million was recognized during the year ended December 31, 2016 and include a non-cash impairment charge of \$1.4 million.

Restructuring charges were as follows (in thousands):

	Three months ended March 31,	
	2017	2016
Employee severance and related costs	\$ 204	—
Contract termination costs	2,353	—
<b>Total restructuring charges</b>	<b>\$ 2,557</b>	<b>—</b>

The accrued restructuring activity for the three months ended March 31, 2017 was as follows (in thousands):

	Employee severance and related costs	Contract Termination Costs	Total
Balance at December 31, 2016	\$ 1,097	\$ 1,565	\$ 2,662
Restructuring charges and adjustments	204	2,353	2,557
Cash payments	(1,219)	(100)	(1,319)
Balance at March 31, 2017	\$ 82	\$ 3,818	\$ 3,900

The total accrued restructuring balance of \$3.9 million has been presented as a current liability within accrued expenses.

### *Employee severance and related costs*

Employee severance and benefits costs recorded in restructuring charges for the three months ended March 31, 2017 included \$0.2 million in accrued retention costs, which are being ratably recognized over the respective employee's service period.

### *Contract termination costs*

Contract termination costs recorded in restructuring charges for the year ended December 31, 2016 of \$1.5 million related to the Company's determination to cease use and not occupy the Company's headquarters and research facility in connection with the lease with the Company entered into in June 2016 (see Note 10). In connection with this determination, the Company recorded a liability of \$1.6 million, which was equal to the fair value of the lease obligation at the cease-use date of November 20, 2016. The Company estimated the liability for the contract termination costs associated with the lease as of the cease-use date based on the discounted present value of the remaining lease payments, considering future estimated sublease income, estimated broker fees and contractual executory costs.

In May 2017, the Company and its landlord entered into the Lease Settlement (defined in Note 12) to terminate the lease described in Note 10 for consideration of approximately \$3.8 million ("the Settlement Amount"). The Lease Settlement is contingent upon the landlord's execution of a new lease with a third party prior to May 30, 2017, which date the landlord may extend, at its sole option, by 60 days. The Company adjusted its liability for contract termination costs to the Settlement

Amount, resulting in an adjustment of \$2.4 million recorded in restructuring charges for the three months ended March 31, 2017. See Note 12 for additional discussion surrounding the termination of the lease.

#### *Asset impairment costs*

Following the discontinuation of research and development activities and corresponding workforce reduction, the Company determined that certain property and equipment was impaired and recognized an impairment charge of \$1.4 million in restructuring expense in the statement of operations for the year ended December 31, 2016. Of the total impairment charge, approximately \$555,000 relates to the impairment of the Lab Equipment which was sold for \$325,000 in February 2017 (see Note 5). In addition, the Company recognized an impairment of \$591,000 during the year ended December 31, 2016 in construction in progress for the Company's planned headquarters and research facility associated with the termination of the lease contract discussed above. Further, following the workforce reduction, the Company sold or donated its remaining office equipment with the exception of nominal office equipment necessary to continue administrative functions and the closure of its Phase 1 clinical trial, resulting in an impairment of furniture, computers and equipment and leasehold improvements of \$256,000 recognized during the year ended December 31, 2016.

## **10. 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 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. In November 2016, following the workforce reduction described in Note 9, the Company determined not to occupy and ceased use of the headquarters and research facility under the Lease. See Note 9 to the financial statements for further information on accounting for the Lease.

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. There have been no draws on the improvement allowance as of March 31, 2017.

Mirna 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 Mirna 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:

<b>Period ending December 31,</b>	<b>Operating Lease</b>
2017 (nine months) \$	450,929
2018	614,855
2019	633,364
2020	652,340
2021 and thereafter	4,565,888
<b>Total</b>	<b>\$ 6,917,376</b>

In May 2017, the Company and its landlord entered into the Lease Settlement (defined in Note 12) to terminate the Lease for consideration of approximately \$3.8 million. See Note 12 for additional discussion surrounding the termination of the Lease.

### ***CPRIT***

In August 2010, the Company entered into a grant contract ("2010 Grant Contract") with the Cancer Prevention and Research Institute of Texas (CPRIT), under which it received a \$10.3 million commercialization award from the State of Texas through CPRIT. CPRIT was established to expedite innovation and commercialization in the area of cancer research and to enhance access to evidence-based prevention programs and services throughout the State of Texas. The award was a three-year award that was funded annually, and the contract terminated on January 31, 2014, subject to the Company's obligations to make

certain payments that survive termination. Under the terms of the award, the Company will be required to pay to CPRIT a portion of the Company's revenues from sales of certain products by the Company, 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 the Company's right, under certain circumstances, to make a one-time payment in a specified amount to CPRIT to buy out such payment obligations. The 2010 grant contract also contains a provision that provides for repayment to CPRIT some amount not to exceed the full amount of the grant proceeds under certain specified circumstances involving relocation of our principal place of business outside Texas.

## 11. 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 March 31,	
	2017	2016
Net loss attributable to common stockholders—basic and diluted	4,977	6,571
Weighted-average number of common shares—basic and diluted	20,850,494	20,830,555
Net loss per share attributable to common stockholders—basic and diluted	\$ (0.24)	\$ (0.32)

As of March 31, 2017 and 2016 the Company has excluded 1,611,321 and 1,915,709 potentially dilutive stock options outstanding, respectively, from the computation of diluted weighted average common shares outstanding, prior to the use of the treasury stock method or if-converted method, because including them would have had an anti-dilutive effect due to the losses reported.

As the Company incurred a net loss for the three months ended March 31, 2017 and March 31, 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.

## 12. Subsequent Events

### *Lease Termination*

In May 2017, the Company and G&I VII Encino Trace II LP (the "Landlord") entered into a Lease Termination Agreement and Release (the "Lease Settlement") to terminate the lease described in Note 10 for consideration of approximately \$3.8 million (the "Settlement Amount"). This agreement is contingent upon the Landlord's execution of a new lease with a third party prior to May 30, 2017 (the "Contingency Date"). The Landlord may extend the Contingency Date, at its sole option, for a period of up to 60 days. The Lease Settlement includes standard representations and warranties and releases both the Company and the Landlord from their mutual obligations under the Lease, provided, for purposes of the Landlord's release, the Company does not file for bankruptcy or otherwise make an assignment for the benefit of creditors within 90 days of the Landlord's receipt of the Settlement Amount.

## 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, 2016, filed with the U.S. Securities and Exchange Commission, or the SEC, on March 15, 2017.*

### 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 that has historically focused on microRNA-based oncology therapeutics, which are short ribonucleic acid, or RNA, molecules, or oligonucleotides.

Our first product candidate, MRX34, was studied as a single agent in a Phase 1 clinical trial. 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 in the trial. 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 was on full clinical hold. Following our suspension of the Phase 1 trial for MRX34 and the FDA's clinical hold on the IND for MRX34, we discontinued development of MRX34 and our microRNA product pipeline and closed our IND.

In November 2016, we discontinued research and development activities to reduce operating expenses while we evaluate our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company. We also initiated a plan in November 2016 to reduce personnel and expenses to preserve capital and further streamline our operations consistent with our decision to discontinue development of MRX34 and our microRNA product pipeline. We expect to devote significant time and resources to identifying and evaluating strategic alternatives, 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.

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. We have not generated any revenue from product sales and, to date, have funded our operations primarily through the private placement of convertible preferred stock, federal and state government grants, offerings of our common stock, and support from our former parent company, Asuragen. From our inception through March 31, 2017, 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 \$5.0 million for the three months ended March 31, 2017. At March 31, 2017, we had an accumulated deficit of \$107.8 million. We expect to continue to incur significant expenses and operating losses. Our net losses may fluctuate significantly from quarter to quarter and from year to year. We anticipate that our expenses will continue to decrease as we discontinued our research and development activities, further reduce headcount, and focus on evaluating our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company.

## **Financial Operations Overview**

### ***Revenue***

We have not generated any revenue from product sales or from collaborations. In the future, we may generate revenue following a potential strategic transaction. Revenue may fluctuate from period to period, and the timing and extent of any future revenue will depend on our ability to consummate a strategic transaction.

### ***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 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.

In September 2016, Mirna announced its decision to close the Phase 1 study of MRX34 and voluntarily halted the enrollment and dosing of patients in the study. Further, in November 2016, the Company discontinued research and development activities to reduce operating expenses.

Research and development costs have been expensed as incurred. In certain circumstances, we have made nonrefundable advance payments to purchase goods and services for future use pursuant to contractual arrangements. In those instances, we deferred and recognized 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 provided 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.

Prior to discontinuing our research and development activities, 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 basis. However, we historically spent the vast majority of our research and development resources on our first product candidate, MRX34, development of which has been stopped.

We anticipate that our research and development expenses will continue to decrease as we discontinued our research and development activities, further reduce headcount, and focus on evaluating our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company.

### *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 expect that general and administrative expenses related to our operations will decrease as a result of the workforce reduction and discontinuance of our research and development activities. These decreases may be offset in whole or in part following the change in our corporate strategy to focus on pursuing potential strategic initiatives to enhance stockholder value.

### **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 our estimates and judgments, including those related to stock-based compensation, clinical trial and pre-clinical study accruals and restructuring charges. 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 three months ended March 31, 2017, 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, 2016, which was filed with the SEC on March 15, 2017.

### **Results of Operations**

#### *Comparison of three months ended March 31, 2017 and 2016:*

	<b>Three Months Ended March 31,</b>		<b>Dollar Change</b>	<b>% Change</b>
	<b>2017</b>	<b>2016</b>		
<b>(in thousands)</b>				
<b>Statement of operations data:</b>				
Operating expenses:				
Research and development	\$ 242	\$ 4,523	\$ (4,281)	(94.6)%
General and administrative	2,264	2,130	134	6.3 %
Restructuring charges	2,557	—	2,557	100.0 %
Interest (income)	(86)	(82)	(4)	4.9 %
Net loss	<u>\$ 4,977</u>	<u>\$ 6,571</u>	<u>\$ (1,594)</u>	<u>(24.3)%</u>

### *Research and Development Expenses*

Research and development expenses were \$0.2 million for the three months ended March 31, 2017 which was a decrease of \$4.3 million or 95%, compared to research and development expenses of approximately \$4.5 million for the three months ended March 31, 2016. The decrease in the three months ended March 31, 2017 was primarily due to the discontinuation of the Company's research and development activities, including development of MRX34 and our microRNA pipeline, and corresponding reduction in force, both of which were implemented in November 2016 to reduce operating expenses and streamline operations while the Company evaluates strategic alternatives.

We anticipate that our research and development expenses will continue to decrease as we discontinued our research and development activities, further reduce headcount, and focus on evaluating our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company.

### *General and Administrative Expenses*

General and administrative expenses were approximately \$2.3 million for the three months ended March 31, 2017, which was an increase of approximately \$0.1 million or 6%, compared to general and administrative expenses of \$2.1 million for the three months ended March 31, 2016. The increase in the three months ended March 31, 2017 was primarily due to approximately \$0.5 million in legal fees for transaction costs related to the Company's evaluation of strategic alternatives. This increase was largely offset by a decline in personnel and operating expenses following the reduction in force implemented in November 2016 to reduce costs and streamline operations while the Company evaluates strategic alternatives.

### *Restructuring charges*

Restructuring charges were approximately \$2.6 million for the three months ended March 31, 2017. The Company and its landlord entered into a Lease Termination Agreement and Release (the "Lease Settlement") to terminate the Company's lease of approximately 23,578 square feet of office and laboratory space entered into in June 2016 for consideration of approximately \$3.8 million. As a result of the Lease Settlement, the Company recognized incremental contract termination costs based on the Settlement Amount of approximately \$2.3 million, which was recorded in restructuring charges during the three months ended March 31, 2017. The remaining restructuring charges primarily related to accrual of retention benefits provided to employees in connection with the reduction in force which took place in November 2016 recognized over the respective employee's service period. There were no restructuring charges during the three months ended March 31, 2016.

## **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 March 31, 2017, we had \$17.1 million of cash and cash equivalents and \$40.4 million invested in marketable securities for a total of \$57.5 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 March 31, 2017, 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.

In addition, certain executive officers are entitled to certain payments if they are terminated without cause or as a result of a change in control. Upon termination without cause, and not as a result of death or disability, each of such officers is entitled to receive payment of base salary for 9 to 12 months following termination of employment and certain officers will be entitled to continue to receive coverage under medical and dental benefit plans for 9 to 12 months or until such officers are covered under a separate plan from another employer. Upon a termination other than for cause or with good reason following a change in control, each of such officers is entitled to receive payment of base salary for 12 to 18 months following termination of employment and 100% to 150% of the executive's target bonus paid in a single cash lump sum. In addition, the officers will be entitled to continue to receive coverage under medical and dental benefit plans for 12 to 18 months or until such officers are covered under a separate plan from another employer.

The following table shows a summary of our cash flows for the three months ended March 31, 2017 and 2016.

	Three Months Ended March 31,	
	2017	2016
	(in thousands)	
Net cash provided by (used in):		
Operating activities	\$ (3,216)	\$ (8,973)
Investing activities	3,880	(27,865)
Financing activities	25	—
Net increase (decrease)	<u>\$ 689</u>	<u>\$ (36,838)</u>

### ***Operating Activities***

Net cash used in operating activities was \$3.2 million and \$9.0 million for the three months ended March 31, 2017 and 2016, respectively. The decrease in overall spending for operating activities of approximately \$5.8 million was due to reduced personnel and operating expenses following the reduction in force and discontinuation of the Company's research and development activities, which occurred in November 2016. Excluding the impact of restructuring charges in the three months ended March 31, 2017, the Company's net loss decreased from \$6.5 million to \$2.4 million for the months ended March 31, 2016 and 2017, respectively, a decrease of \$4.1 million. In addition, the Company made an up-front licensing payment of \$1.6 million reflected in operating activities during the three months ended March 31, 2016.

### ***Investing Activities***

Net cash used in investing activities for the periods presented relates primarily to the purchases and maturities of marketable securities during the three months ended March 31, 2017 and 2016. For the three months ended March 31, 2017, the Company had net inflows related to marketable securities of \$3.6 million, as the maturities of securities exceeded purchases during the period, whereas for the three months ended March 31, 2016, the Company had net outflows \$27.7 million, as the Company began investing the proceeds from the Company's initial public offering during the first quarter of 2016.

### ***Financing Activities***

Net cash provided by financing activities was approximately \$25,000 for the three months ended March 31, 2016, which was attributable to proceeds received from the exercise of stock options.

### **Contractual Obligations and Commitments**

The following table presents payments due under the Company's contractual obligations as of March 31, 2017:

	Total	Payments Due by Period			
		Less than 1 Year	1-3 Years	3-5 Years	Over 5 Years
Operating Lease	\$ 6,917,376	\$ 601,239	\$ 1,257,415	\$ 1,334,037	\$ 3,724,685
Other	32,694	32,694	—	—	—
	<u>\$ 6,950,070</u>	<u>\$ 633,933</u>	<u>\$ 1,257,415</u>	<u>\$ 1,334,037</u>	<u>\$ 3,724,685</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 March 31, 2017, we had cash and cash equivalents of \$17.1 million and short-term marketable securities of \$40.4 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, 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 March 31, 2017. 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 submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, or persons performing similar functions, 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 March 31, 2017, 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 have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II OTHER INFORMATION

### Item 1. Legal Proceedings

We are not currently 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 business to date has been almost entirely dependent on the success of MRX34, and we have decided to discontinue further development of MRX34 and our microRNA product pipeline 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 SAEs and the IND for MRX34 was placed on full clinical hold. In November 2016, we discontinued research and development activities to reduce operating expenses while we evaluate our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company. 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 can be no assurance that this transaction would enhance shareholder value. There also can be no assurance that we will conduct further drug research or development activities in the future.

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.

***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) 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; (ii) potential litigation against us, and other various claims and legal actions arising in the ordinary course of business; and (iii) 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.

***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. In November 2016, we discontinued research and development activities to reduce operating expenses while we evaluate our strategic alternatives with a goal to enhance stockholder value, including the possibility of a merger or sale of the Company. To date, we have derived all of our funding from our collaboration with our former parent company, Asuragen, Inc., or Asuragen, private and public placements of our capital stock and government grants for research and development. Our net loss for the three months ended March 31, 2017 was \$5.0 million. Since inception, we have incurred net losses leading to an accumulated deficit of approximately \$107.8 million as of March 31, 2017.

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. None of our product candidates are in clinical development and, in November 2016, we discontinued our research and development activities relating to our product candidates that were in preclinical 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.

### **Risks Related to Product Development and Commercialization**

***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.

We have product liability insurance that we feel is appropriate for our stage of development, which covers clinical trials in the United States, for up to \$1 million per occurrence, up to an aggregate limit of \$5 million; however, our insurance may be insufficient to reimburse us for any expenses or losses we may suffer. Our product liability insurance policy for clinical trials completed in the United States expires on December 31, 2017. In addition, we have product liability insurance, which covers clinical trials in the Republic of Korea, for up to KRW 625,000,000 per occurrence, or approximately \$500,000, up to an aggregate limit of KRW 2,500,000,000 or approximately \$2,000,000. Our product liability insurance policy for clinical trials completed in the Republic of Korea includes one additional year of coverage expiring on October 11, 2017. 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;
- 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.

***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.***

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:

- potentially 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. In addition, the 2010 grant contract also contains a provision that provides for repayment to CPRIT some amount not to exceed the full amount of the grant proceeds under certain specified circumstances involving relocation of our principal place of business outside Texas. See also "Business-Strategic Partnerships and Licenses" 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.

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.

## 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. Effective in December 2016, we terminated the employment of Jon Irvin, our Vice President of Finance, in connection with our restructuring 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 ability to execute our strategic plan.

***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 (if any) 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 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 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. Alternatively, we may 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 \$1.07 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 \$1.07 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 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.”

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 were not 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. 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, if we resume our research and development activities and integral parties in our supply chain are geographically concentrated and operating from single sites, this would increase their vulnerability to natural disasters or other sudden, unforeseen and severe adverse events. If such an event were to affect such parties in our supply chain, it could have a material adverse effect on our business.

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

***If we are sued for infringing the patent rights or misappropriating the trade secrets of third parties, such litigation could be costly and time consuming.***

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 product 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.

If we are found to infringe a third party's patent rights, 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 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.

***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, if we resume our research and development activities which have been discontinued. These include our exclusive cross-license agreement with Asuragen, our exclusive licenses from 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 (if any) would 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 Partnerships and Licenses" 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, Marina, the University of Zurich and Rosetta Genomics.

As we have done previously, if we commence research and development of product candidates, we may need to obtain licenses from third parties to advance research or allow commercialization of 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 resume our research and development activities. 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.

***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 University, or 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 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.

***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 stockholders in the future;
- 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 Global Market. If our stock trades at bid prices of less than \$1.00 for a period in excess of 30 consecutive business days, NASDAQ could send a deficiency notice to us for not remaining in compliance with the minimum bid listing standards. During the first quarter of fiscal year 2017, 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 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 March 31, 2017, our officers and directors, together with holders of 5% or more of our outstanding common stock and their respective affiliates, beneficially own approximately 69.8% 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.

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 March 31, 2017, we have a total of 20,856,693 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 March 31, 2017, 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 the 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 13.6 million shares of our common stock at March 31, 2017 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;
- our execution of any collaborative, licensing or similar arrangements, and the timing of payments we may make 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.

***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 \$2.5 million at March 31, 2017 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 our operations. 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 \$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 discontinued further research and development activities to reduce operating expenses while it evaluates these opportunities. We currently expect to use the remaining net proceeds from our initial public offering primarily for working capital and other general corporate purposes, which include our activities to evaluate and pursue strategic alternatives.

### ***Issuer Purchases of Equity Securities***

None.

## **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 1.01 - Entry into a Material Definitive Agreement” and “Item 1.02 - Termination of a Material Agreement” of Form 8-K.

On May 5, 2017, the Company entered into a Lease Termination Agreement and Release (the “Lease Settlement”) with G&I VII Encino Trace II LP (the “Landlord”) to terminate that certain lease agreement dated June 24, 2016 by and between the Company and the Landlord (the “Lease Agreement”) for the Company’s lease of approximately 23,578 square feet of real property located at 5707 Southwest Parkway, Suite 100, Austin, Texas 78735 (the “Premises”). Pursuant to the Lease Settlement, the Company has agreed to pay the Landlord approximately \$3.8 million (the “Settlement Amount”) within three business days of May 5, 2017.

The Lease Settlement provides for the Company’s release of claims against the Landlord and its affiliates and, upon the Landlord’s receipt of the Settlement Amount, the Landlord’s release of claims against the Company, provided the Company does not file for bankruptcy or otherwise make an assignment for the benefit of creditors within 90 days of the Landlord’s receipt of the Settlement Amount.

The termination of the Lease Agreement is to be effective as of, and contingent upon, the Landlord’s execution of a new lease for the Premises with a third party on or before May 30, 2017, which date the Landlord, at its sole option, may extend for up to 60 days. If the new lease is not executed within such period, the Lease Settlement will be considered null and void and the Landlord will be required under the terms of the Lease Settlement to return the Settlement Amount to the Company.

The foregoing description of the Lease Settlement is not complete and is qualified in its entirety by reference to the full text of the Lease Settlement, which will be filed as an exhibit to the Company's Quarterly Report on Form 10-Q for the three months ending June 30, 2017.

**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 Mirna 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: May 9, 2017

/s/ Paul Lammers

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

Date: May 9, 2017

/s/ Alan Fuhrman

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Alan Fuhrman  
Chief Financial Officer  
(Principal Financial & Accounting 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 Mirna 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 9, 2017

/s/ PAUL LAMMERS

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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 Mirna 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 9, 2017

/s/ ALAN FUHRMAN

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Alan Fuhrman

Chief Financial Officer

(Principal Financial & Accounting 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 Mirna Therapeutics, Inc. (the "Company") on Form 10-Q for the fiscal quarter ended March 31, 2017, 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: May 9, 2017

/s/ PAUL LAMMERS

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

Chief Executive Officer

(Principal Executive Officer)

/s/ ALAN FUHRMAN

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Alan Fuhrman

Chief Financial Officer

(Principal Financial & Accounting Officer)