March 29, 2016

Mirna Therapeutics Provides Operational Update and Reports Financial Results for Fourth Quarter and Full Year Ended December 31, 2015

Management to host conference call and webcast today at 5 p.m. Eastern

AUSTIN, Texas--(BUSINESS WIRE)-- Mirna Therapeutics, Inc. (Nasdaq:MIRN), a clinical stage biopharmaceutical company developing a broad pipeline of microRNA-based oncology therapeutics, today reported financial results for the fourth quarter and full year ended December 31, 2015, and provided an update on the Company's recent progress.

"Mirna had a highly productive 2015 as we continued to advance the first microRNA therapeutic in clinical development in cancer," commented Paul Lammers, M.D., M.Sc., Mirna's President and CEO. "Our lead microRNA product candidate, MRX34, has demonstrated clinically significant responses in multiple cancers, and we are preparing to advance it into two Phase 2 studies late this year. We also continued to broaden our understanding of its unique mechanism capable of inhibiting multiple oncogenic and immune-mediated pathways known to drive tumor growth."

Dr. Lammers continued, "On the corporate front, we raised a total of over $100 million from an IPO and two private placements during the year and strengthened our management team, leaving us well-positioned to advance our clinical development strategy and fully leverage our microRNA technologies for the treatment of cancers."

MRX34 Program Updates

- **Achieved confirmed partial responses in multiple advanced cancers.** In the ongoing Phase 1 trial of MRX34, a confirmed partial response (PR) using the Response Evaluation Criteria in Solid Tumors (RECIST) was observed in a patient with advanced renal cell carcinoma, in addition to the previously announced confirmed PRs in patients with advanced acral melanoma and hepatocellular carcinoma. In addition, a number of patients with solid tumors achieved long-term stable disease during treatment with MRX34. The Company currently expects to provide a further clinical update from this trial in mid-2016 and top-line data in mid-2017.
- **MRX34 to enter Phase 2 clinical studies.** The Company expects to initiate two Phase 2 clinical studies by the end of 2016. The two studies will evaluate MRX34 in patients with melanoma and renal cell carcinoma. These tumor types were chosen based on clinical results generated to date in the ongoing Phase 1 trial. Each Phase 2 trial is expected to enroll approximately 30 patients to further characterize the safety and efficacy of MRX34 in these cancer types. The Company currently plans to provide a clinical update from these studies in the second half of 2017.
- **MRX34 Phase 1b translational medicine study planned.** This clinical study is expected to include serial tumor biopsies and aims to develop deeper insights into the mechanism of action of MRX34 in melanoma patients, including biomarkers related to pharmacodynamic activity and therapeutic response. The study is expected to begin in late 2016.
- **Expansion of development program to include combination and additional microRNA therapies.** Combination preclinical studies are planned and ongoing in models of non-small cell lung cancer. The Company expects that, if positive results are obtained in these preclinical studies, the combination program may progress toward clinical testing in 2017. Preclinical studies are also ongoing to support selection of a second microRNA product candidate from the Company's pipeline, with an Investigational New Drug (IND) application planned in late 2017.

2015 Highlights

- **Presented results from the Phase 1 study at the Annual Meeting of the American Association for Cancer Research (AACR).** In April, data were presented including a molecular analysis of white blood cells from patients treated with MRX34 in the Phase 1 clinical trial showing a dose-dependent repression of several key oncogenes previously identified as direct miR-34 targets including FOXP1, BCL2, HDAC1 and CTNNB1. These data suggest delivery of miR-34 into human white blood cells and engagement of several biological targets of the miRNA.
- **Data published in *Journal of the National Cancer Institute (JNCI).*** The Company published results supporting potential immune-related mechanisms for anti-cancer activity of MRX34. Specifically, the data indicate that miR-34 can regulate anti-tumor immune functions by repressing PD-L1 (programmed death receptor ligand 1), an immune checkpoint signaling molecule that is upregulated by many tumor cells to escape the surveillance of the body's immune system. The preclinical study was conducted in collaboration with researchers from the University of Texas

MD Anderson Cancer Center.

Corporate Activities

- **Raised more than $100 million in funding.** The Company completed its initial public offering (IPO) in October 2015, raising gross proceeds of $48.7 million. Concurrent with the IPO, the Company closed a private placement with the Cancer Prevention and Research Institute of Texas (CPRIT) for additional gross proceeds of $16.8 million. In March and April 2015, Mirna raised $41.8 million in a Series D private financing.
- **Strengthened management team.** The Company appointed Miguel Barbosa, Ph.D., as Executive Vice President and Chief Scientific Officer, and Alan Fuhrman as Chief Financial Officer. Dr. Barbosa recently served in roles as Head of Immunology Research at Janssen Research & Development and as Executive in Residence, Therapeutic Innovation at Johnson & Johnson Innovation. Mr. Fuhrman most recently was Chief Financial Officer of Ambit Biosciences.

Fourth Quarter and Full Year 2015 Financial Results

- **Cash Position and Guidance**: Cash and cash equivalents totaled $89.7 million as of December 31, 2015, compared to $9.3 million as of December 31, 2014. The Company has no debt. Based on the current operating plan, the Company expects that current cash resources will be sufficient to meet operating requirements into 2018.
- **Research and Development Expenses**: Research and development expenses were $6.3 million and $18.9 million for the fourth quarter and year ended December 31, 2015, compared to $3.5 million and $10.5 million during the comparable periods in 2014. The increase in research and development expenses in 2015 was primarily due to increased clinical trial costs related to the ongoing Phase 1 clinical trial, including a higher number of patients, additional investigator sites and additional drug costs, increased headcount and personnel costs, and increased intellectual property and licensing costs. The increases were partially offset by higher grant reimbursements.
- **General and Administrative Expenses**: General and administrative expenses were $2.5 million and $6.1 million for the fourth quarter and year ended December 31, 2015, compared to $0.9 million and $3.4 million during the comparable periods in 2014. The increase in general and administrative expenses was primarily due to increased personnel related expenses, and higher outside professional and consulting costs, a majority of which are costs to comply with public company operating and reporting requirements.
- **Net Loss**: Net loss was $8.8 million for the fourth quarter of 2015 compared to $4.4 million in the comparable period in 2014. The results included non-cash, stock-based compensation charges of approximately $424,000 in the three months ended December 31, 2015 and approximately $121,000 in the comparable period in 2014.

  Net loss was approximately $25.0 million for the year ended December 31, 2015, compared to $15.8 million for the comparable period in 2014. The results included non-cash, stock-based compensation charges of approximately $985,000 in the year ended December 31, 2015 and approximately $408,000 in the comparable period in 2014.

Conference Call and Webcast

Mirna management will host a webcast and conference call at 5:00 p.m. Eastern today to discuss the financial results. To access the call, participants should dial 877-407-9079 (US & Canada) or 201-493-6746 (international) at least 10 minutes prior to the start of the call. The call will be webcast live and may be accessed from the Events & Presentations section of the Company's website. An archived version of the webcast will be available for replay for up to 30 days following the event.

About Mirna Therapeutics, Inc.

Mirna is a clinical-stage biopharmaceutical company developing a broad pipeline of microRNA-based oncology therapeutics and is the first to establish clinical proof-of-concept for a microRNA replacement therapy for cancer. Mirna's lead product candidate, MRX34, a mimic of naturally occurring microRNA-34 (miR-34), is currently being studied in a Phase 1 clinical trial in patients with primary liver cancer, advanced solid tumors and hematological malignancies. miR-34 is one of the most widely published microRNAs and is considered a key regulator of multiple oncogenes across key oncogenic pathways, with the capacity to regulate more than 30 different oncogenes and repress the immune checkpoint signaling molecule PD-L1. The potential capacity to simultaneously affect multiple pathways and processes that are critical to cancer cell viability may make mimics of tumor suppressor microRNAs potent anti-cancer agents and less susceptible to drug resistance. Mirna plans to develop MRX34 as a monotherapy and in combination with other therapeutic modalities, such as targeted therapies and immuno-oncology agents. The Company was founded in 2007 and is located in Austin, Texas.

For more information, visit www.mirnarx.com.

Forward-Looking Statements

To the extent that statements contained in this press release are not descriptions of historical facts regarding Mirna, they are forward-looking statements reflecting the current beliefs and expectations of management made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, including statements regarding our pre-clinical and clinical activity and results of our clinical development program, including the projected timing and nature of a potential Phase 1b translational medicine study and Phase 2 studies of MRX34, our pre-clinical combination therapy studies of MRX34 and pre-clinical studies and a potential filing of an IND for a potential second product candidate; our ability to understand the anti-cancer properties of MRX34; and our belief that we are well-positioned to advance our clinical development strategy. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development program, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, including the outcomes of clinical trials, the regulatory approval process, our substantial dependence on MRX34, our commercialization plans and efforts and other matters that could affect the availability or commercial potential of our product candidates. Mirna undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to our business in general, see our Quarterly Report on Form 10-Q filed with the U.S. Securities and Exchange Commission (SEC) on November 13, 2015 and our Annual Report on Form 10-K that we expect to file with the SEC on or about March 29, 2016.

Mirna Therapeutics, Inc.
Condensed Balance Sheet
(in thousands, except per share data)

	December 31, 2015	December 31, 2014
Assets		
Current Assets:		
Cash and cash equivalents	$ 89,713	$ 9,319
Grant reimbursement and other receivables	36	155
Prepaid expenses and other current assets	793	143
Total current assets	90,542	9,617
Property and equipment, net	375	116
Deferred offering costs	—	92
Total assets	$ 90,917	$ 9,825
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current Liabilities:		
Accounts payable	$ 3,687	$ 871
Accrued expenses	2,214	1,628
Total liabilities	5,901	2,499
Commitments and contingencies		
Convertible preferred stock, $0.001 par value; 0 shares and 84,000,783 shares authorized at December 31, 2015 and 2014, respectively;		
Series A, Series B, Series B-1, Series C and Series D: 0 and 5,599,939 shares issued and outstanding at December 31, 2015 and 2014, respectively; aggregate liquidation preference of $0 and $55.3 million at December 31, 2015 and 2014, respectively	—	55,277
Stockholders' Equity (Deficit):		
Preferred stock, $0.001 par value, 5,000,000 and 0 shares authorized at December 31, 2015 and 2014; 0 shares outstanding at December 31, 2015 and 2014	—	—
Common stock, $0.001 par value; 250,000,000 and 95,000,000 shares authorized at December 31, 2015 and 2014, respectively; 20,830,555 and 83,325 shares issued and outstanding at December 31, 2015 and 2014, respectively	21	—
Additional paid in capital	161,518	—
Accumulated deficit	(76,523)	(47,951)
Total stockholders' equity (deficit)	85,016	(47,951)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 90,917	$ 9,825

Mirna Therapeutics, Inc.
Condensed Statements of Operations

	For the Three Months Ended December 31,		For the Year Ended December 31,	
	2015	2014	2015	2014
	(unaudited)	(unaudited)		
Operating expenses:				
Research and development	$ 6,363	$ 3,501	$ 18,947	$ 10,545
General and administrative	2,462	877	6,080	3,369
Write-off of offering costs	—	—	—	1,920
Total operating expenses	8,825	4,378	25,027	15,834
Other (income):				
Interest (income)	(36)	—	(44)	—
Total other (income)	(36)	—	(44)	—
Net loss	(8,789)	(4,378)	(24,983)	(15,834)
Less: Accretion and dividends on convertible preferred stock	(101)	(712)	(4,320)	(2,824)
Net loss attributable to common stockholders	$ (8,890)	$ (5,090)	$ (29,303)	$ (18,658)

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