August 15, 2016

Mirna Therapeutics Reports Second Quarter 2016 Financial Results and Program Updates

AUSTIN, Texas--(BUSINESS WIRE)-- Mirna Therapeutics, Inc. (Nasdaq:MIRN), a clinical stage biopharmaceutical company developing a pipeline of microRNA-based oncology therapeutics, today reported financial results for the second quarter of 2016 and provided an update on recent developments.

MRX34 CLINICAL PROGRAM AND SECOND QUARTER UPDATE

- **Presented clinical data of MRX34 at ASCO 2016.** In an oral presentation, investigators reported on the final dose-escalation results from the first-in-human Phase 1 trial of MRX34, highlighting the safety profile, pharmacodynamic evidence of activity, and multiple clinical responses in cancer patients with a variety of advanced solid tumors.

  Data highlights included four confirmed partial responses for up to 50+ weeks in duration in patients with late-stage, metastatic hepatocellular carcinoma cancer or HCC (liver cancer), renal cell carcinoma or RCC (kidney cancer) and acral melanoma (a rare and difficult-to-treat form of skin cancer). Stable disease was also observed in an additional 15 patients for more than four cycles (approximately three months) of therapy, including a small cell lung cancer (SCLC) patient who showed stable disease for more than one year on MRX34 as fourth line therapy.

  As of the ASCO presentation, three patients had experienced possible immune-mediated serious adverse events (SAEs) after receiving MRX34. These previously reported events included enterocolitis, systemic inflammatory response syndrome, and pneumonitis/colitis. The first two patients recovered; the patient experiencing pneumonitis/colitis subsequently died.

- **Clinical Update.** In early August, a recently enrolled acral melanoma patient experienced an SAE of increased ALT and AST liver function tests, determined likely to be due to acute hepatitis, with subsequent liver failure leading to death. The event was deemed possibly related to MRX34 and reported to the FDA and Korean regulatory authority.

  The timing and pattern of response to treatment with MRX34 and the associated safety profile suggest a potential immune component to MRX34 activity.

- **Plan to continue the expansion phase of the ongoing Phase 1 study.** Enrollment efforts are focused on patients with cancer types where confirmed responses have been observed as well as others where miR-34 has shown biological relevance. The Company had planned to initiate Phase 2 trials in RCC and melanoma by the end of 2016. However, it is now planned to let the results from the Phase 1 expansion cohorts and the translational medicine study guide the next steps in development of MRX34.
- **Preparing to launch translational medicine trial in late 2016.** This study is intended to develop deeper insights into the mechanism of action of MRX34 in metastatic melanoma patients and identify potential biomarkers of drug activity and treatment response. The study is planned to include serial tumor biopsies as well as liquid biopsies for cell-free DNA and exosomal RNA analysis.
- **Preclinical studies underway, evaluating potential of combination regimens to enhance effectiveness of standard cancer therapies.** Mirna researchers presented *in vitro* findings at the American Association for Cancer Research (AACR) 2016 annual meeting demonstrating the synergistic anticancer effects between MRX34 and platinum and other commonly used cytotoxic chemotherapy drugs across a range of non-small cell lung cancer (NSCLC) cell lines. Synergistic anticancer effects were also shown between MRX34 and tyrosine kinase inhibitors, suggesting the potential for combination therapies that include MRX34 with other standard of care cancer therapeutics.

CORPORATE UPDATES

- **Appointed Dr. Vincent J. O'Neill as the Company's Chief Medical Officer.** Dr. O'Neill, a medical oncologist, joined Mirna with 15 years of therapeutic and diagnostic product development experience, most recently serving as Chief Medical Officer at Exosome Diagnostics. Previous roles included senior leadership positions at Sanofi, Genentech and GlaxoSmithKline.

- **Appointed Dr. Perry Nisen to the Board of Directors.** Dr. Nisen is currently the Chief Executive Officer and

Donald Bren Chief Executive Chair of the Sanford Burnham Prebys Medical Discovery Institute. Previously, he served as Senior Vice President of Science and Innovation at GlaxoSmithKline (GSK), where he was integral to the discovery, development, and commercialization of a vast portfolio of oncology drugs. Earlier roles included Senior Vice President and Oncology Therapy Area Head at GSK and Divisional Vice President of Cancer Research and Oncology Development at Abbott Laboratories.

SECOND QUARTER 2016 FINANCIAL RESULTS

- **Cash Position and Guidance:** Cash, cash equivalents, and marketable securities totaled $72.6 million as of June 30, 2016, compared to $89.7 million as of December 31, 2015. 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 in the second quarter of 2016 were $3.7 million and $8.2 million, respectively, for the three and six months ended June 30, 2016, compared to $4.5 million and $7.9 million during the comparable periods in 2015. The decrease for the three months ended June 30, 2016 compared to the same period in 2015 was primarily attributable to higher costs associated with our Phase 1 clinical trial for our lead product candidate MRX34, specifically adding additional sites and upfront drug costs which were incurred in 2015. This decrease in 2016 was partially offset by an increase in employee compensation, benefits and stock compensation.

  The increase for the six months ended June 30, 2016 is primarily attributable to higher compensation, benefits and stock compensation expense due to a higher headcount. The increase was largely offset by higher costs associated with our Phase 1 trial in the prior year.

- **General and Administrative Expenses:** General and administrative expenses in the second quarter of 2016 were $2.0 million and $4.2 million, respectively, for the three and six months ended June 30, 2016, compared to $1.2 million and $2.1 million during the comparable periods in 2015. The increase in general and administrative expenses was primarily attributable to increased employee compensation expense due to a higher headcount and higher outside professional and consulting costs, the majority of which were costs to comply with public company operating and reporting requirements.
- **Net Loss:** Net loss was approximately $5.6 million for the second quarter of 2016 and $12.2 million for the six months ended June 30, 2016, compared to a net loss of $5.7 million and $10.0 million for the comparable periods in 2015. The results included non-cash, stock-based related compensation charges of $243,000 and $689,000 for the second quarter and six months ended June 30, 2016 and $217,000 and $351,000 for the comparable periods in 2015.

About Mirna Therapeutics, Inc.

Mirna is a clinical stage biopharmaceutical company developing a pipeline of microRNA-based oncology therapeutics and is the first to bring a synthetic microRNA mimic into clinical development for the treatment of cancer. Mirna's lead product candidate, MRX34, a mimic of naturally occurring microRNA-34 (miR-34), is being studied in a Phase 1 clinical trial which has included 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 immune checkpoint signaling molecules, including 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 an important new class of anti-cancer agents. Mirna plans to develop MRX34 as a monotherapy and in combination with other therapies. 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 plans for MRX34 development; our plans to let the results from the Phase 1 expansion cohorts and the translational medicine study guide the next steps in development of MRX34; our plans to continue the expansion phase of the ongoing Phase 1 study; our plans to launch a translational medicine trial in late 2016; and our expectation that current cash resources will be sufficient to meet operating requirements into 2018. 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 and the risk that our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval. We undertake 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 Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission (SEC) on March 30, 2016 and our Quarterly Report on Form 10-Q, expected to be filed with the SEC on or about August 15, 2016.

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

	June 30, 2016 (unaudited)	December 31, 2015
Assets		
Current Assets:		
Cash and cash equivalents	$ 30,796	$ 89,713
Marketable securities	41,756	—
Grant reimbursement and other receivables	—	—
Prepaid expenses and other current assets	947	829
Total current assets	73,499	90,542
Property and equipment, net	1,237	375
Restricted cash	2,430	—
Total assets	$ 77,166	$ 90,917
Liabilities and Stockholders' Equity (Deficit)		
Current Liabilities:		
Accounts payable	$ 1,462	$ 3,687
Accrued expenses	2,193	2,214
Total liabilities	3,655	5,901
Commitments and contingencies		
Stockholders' Equity (Deficit):		
Preferred stock, $0.001 par value, 5,000,000 shares authorized at June 30, 2016 and December 31, 2015; 0 shares outstanding at June 30, 2016 and December 31, 2015	—	—
Common stock, $0.001 par value; 250,000,000 shares authorized at June 30, 2016 and December 31, 2015; 20,835,868 and 20,830,555 shares issued and outstanding at June 30, 2016 and December 31, 2015, respectively	21	21
Additional paid in capital	162,216	161,518
Accumulated other comprehensive income	6	—
Accumulated deficit	(88,732)	(76,523)
Total stockholders' equity	73,511	85,016
Total liabilities and stockholders' equity	$ 77,166	$ 90,917

Mirna Therapeutics, Inc.
Condensed Statements of Operations and Comprehensive Loss (unaudited)
(in thousands)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Operating expenses:				
Research and development	$ 3,682	$ 4,499	$ 8,205	$ 7,901
General and administrative	2,049	1,185	4,179	2,062
Total operating expenses	5,731	5,684	12,384	9,963

Other income:				
Interest income	93	—	175	—
Total other income	93	—	175	—
Net loss	$ (5,638)	$ (5,684)	$(12,209)	$ (9,963)
Less: Accretion and dividends on convertible preferred stock	—	(1,544)	—	(2,662)
Net loss attributable to common stockholders	$ (5,638)	$ (7,228)	$(12,209)	$(12,625)

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