



May 12, 2016

## Mirna Therapeutics Reports First Quarter 2016 Financial Results and Program Updates

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 first quarter of 2016 and provided an update on recent developments.

"During the first quarter we continued to make progress advancing our lead product candidate MRX34, the first microRNA therapeutic in clinical development in cancer," commented Paul Lammers, M.D., M.Sc., Mirna's President and CEO. "To date, MRX34 has produced clinically significant responses in patients with various types of late-stage cancers, demonstrating its ability to affect multiple pathways involved in cancer growth and immune evasion. We are continuing to advance MRX34 toward Phase 2 in late 2016 and also pushing ahead with our preclinical program to study its potential in combination with other cancer drugs."

Dr. Lammers continued, "On the corporate front, we were pleased to welcome Peter Greenleaf, Chief Executive Officer of Sucampo Pharmaceuticals, to our Board of Directors, and Dr. Vincent J. O'Neill as our new Chief Medical Officer. We look forward to their unique contributions as we advance our clinical development strategy in the promising new field of microRNA therapeutics."

### FIRST QUARTER AND MRX34 PROGRAM UPDATES

- | **Update from MRX34 Phase 1 trial planned at ASCO.** The Company expects to present additional clinical data from the ongoing Phase 1 trial at the American Society of Clinical Oncology (ASCO) annual meeting in early June. To date, MRX34 has demonstrated compelling clinical results as a single agent therapy, including confirmed partial responses in patients with renal cell carcinoma, acral melanoma, and hepatocellular carcinoma. Additionally, several patients with solid tumors have achieved long-term stable disease during treatment with MRX34. These responses were observed in cancer patients with advanced Stage 4 metastatic disease, whose cancer had progressed after previously receiving multiple rounds of therapy. Top-line data from this study are expected in 2017.
- | **Phase 1b translational medicine trial on track to begin in late 2016.** This study will include serial tumor biopsies and is intended to develop deeper insights into the mechanism of action of MRX34 in melanoma patients and identify potential biomarkers of drug activity and treatment response.
- | **MRX34 expected to begin Phase 2 by end of 2016.** Studies in renal cell carcinoma and melanoma patients are being planned based on the responses observed to date in the ongoing Phase 1 trial, and on the high unmet medical need despite the availability of new therapies.
- | **Preclinical studies ongoing of combination regimens with potential to enhance effectiveness of standard cancer therapies.** At the American Association for Cancer Research (AACR) in April, Mirna researchers presented *in vitro* findings that demonstrated 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. These results suggest a broad potential for combination of MRX34 with other standard of care drug classes.

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.

### CORPORATE UPDATES

- | **Further strengthened management** with the appointments of Peter Greenleaf to the Company's Board of Directors and Dr. Vincent J. O'Neill to the role of Chief Medical Officer. Mr. Greenleaf, an industry veteran with over 20 years of experience in drug development and commercialization, including three years as President of Medimmune, currently serves as the Chief Executive Officer of Sucampo Pharmaceuticals. Dr. O'Neill is a medical oncologist with 15 years of therapeutic and diagnostic product development experience, and has held senior leadership roles at several global pharmaceutical companies, including Sanofi, Genentech and GlaxoSmithKline.

### FIRST QUARTER 2016 FINANCIAL RESULTS

- | **Cash Position and Guidance:** Cash, cash equivalents, and marketable securities totaled \$80.6 million as of March

31, 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 were approximately \$4.5 million for the three months ended March 31, 2016 as compared to \$3.4 million for the same period in 2015. The increase was primarily attributable to increased employee compensation expense due to a higher headcount to support the advancement of the Company's clinical and preclinical development programs, as well as increases in manufacturing, clinical, and intellectual property costs.
- General and Administrative Expenses:** General and administrative expenses were approximately \$2.1 million for the three months ended March 31, 2016 as compared to approximately \$0.9 million for the same period 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 \$6.6 million for the first quarter of 2016 compared to \$4.3 million in the comparable period in 2015. The results included non-cash, stock-based compensation charges of approximately \$447,000 in the three months ended March 31, 2016 and approximately \$134,000 in the same period in 2015.

### 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](http://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 preclinical and clinical activity, including regarding our current or potential Phase 1, 1b and 2 studies of MRX34, and the selection of a second product candidate; and our beliefs regarding the sufficiency of our current cash resources. 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. 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 May 12, 2016.*

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

	<u>March 31,</u> <u>2016</u>	<u>December 31,</u> <u>2015</u>
	(unaudited)	
<b>Assets</b>		
Current Assets:		
Cash and cash equivalents	\$ 52,875	\$ 89,713

Marketable securities	27,713	—
Grant reimbursement and other receivables	168	36
Prepaid expenses and other current assets	853	793
Total current assets	81,609	90,542
Property and equipment, net	516	375
Total assets	<u>\$ 82,125</u>	<u>\$ 90,917</u>
<b>Liabilities and Stockholders' Equity</b>		
Current Liabilities:		
Accounts payable	\$ 1,239	\$ 3,687
Accrued expenses	1,985	2,214
Total liabilities	3,224	5,901
Commitments and contingencies		
Stockholders' Equity (Deficit):		
Preferred stock, \$0.001 par value, 5,000,000 shares authorized at March 31, 2016 and December 31, 2015; 0 shares outstanding at March 31, 2016 and December 31, 2015	—	—
Common stock, \$0.001 par value; 250,000,000 shares authorized at March 31, 2016 and December 31, 2015; 20,830,555 shares issued and outstanding at March 31, 2016 and December 31, 2015	21	21
Additional paid in capital	161,965	161,518
Accumulated other comprehensive income	9	—
Accumulated deficit	(83,094)	(76,523)
Total stockholders' equity	78,901	85,016
Total liabilities and stockholders' equity	<u>\$ 82,125</u>	<u>\$ 90,917</u>

**Mirna Therapeutics, Inc.**

**Condensed Statements of Operations and Comprehensive Loss (unaudited)**  
(in thousands, except per share data)

	<b>Three Months Ended</b>	
	<b>March 31,</b>	
	<b>2016</b>	<b>2015</b>
Operating expenses:		
Research and development	\$ 4,523	\$ 3,402
General and administrative	2,130	877
Total operating expenses	6,653	4,279
Other income:		
Interest income	82	—
Total other income	82	—
Net loss	<u>\$ (6,571)</u>	<u>\$ (4,279)</u>
Less: Accretion and dividends on convertible preferred stock	—	(1,118)
Net loss attributable to common stockholders	<u>\$ (6,571)</u>	<u>\$ (5,397)</u>

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Source: Mirna Therapeutics, Inc.

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