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Mirna Therapeutics Announces Clinical Data on its Lead microRNA Therapeutic Presented at ASCO

Oral presentation highlights safety profile of MRX34, microRNA target engagement, and multiple clinical responses in cancer patients

CHICAGO--(BUSINESS WIRE)-- Mirna Therapeutics, Inc. (Nasdaq: MIRN), a clinical stage biopharmaceutical company developing a broad pipeline of microRNA-based oncology therapeutics, today announced the presentation of clinical data at the 52nd American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago. Investigators reported on the clinical activity of an ongoing, dose-finding Phase 1 trial of MRX34 (miR-34 mimic), Mirna's lead microRNA therapeutic, in patients with a variety of advanced solid tumors.

In an oral presentation (Abstract # 2508 "MRX34, a Liposomal miR-34 Mimic, in Patients with Advanced Solid Tumors: Final Dose-Escalation Results from a First-in-Human Phase 1 Trial of microRNA Therapy"), David S. Hong, M.D., study author and Deputy Chair, Department of Investigational Cancer Therapeutics, Division of Cancer Medicine at The University of Texas MD Anderson Cancer Center, presented results showing MRX34's impact on a broad number of oncogenes and immune pathways, and on the investigational therapy's safety profile and clinical activity.

"The potential of microRNA therapeutics to simultaneously repress multiple oncogenic and immune-evasion pathways represents an exciting new approach to treating cancer," commented Dr. Hong. "In this early study, we are seeing compelling anti-cancer activity, including tumor shrinkage with notable durations of response, supporting further clinical study of MRX34."

Data highlights include:

- Manageable safety profile of MRX34 when administered at the maximum tolerated dose (MTD) with dexamethasone.
- Broad, dose-dependent microRNA target engagement with MRX34, including delivery to tumor sites in patients.
- Four confirmed partial responses (PRs) for up to 54 weeks in duration. This includes patients with late-stage, metastatic cancers including hepatocellular carcinoma (liver cancer), renal cell carcinoma (kidney cancer) and acral melanoma (skin cancer). Timing of these responses and the safety profile observed suggest a potential immune component to MRX34 antitumor activity.
- Stable disease in an additional 15 patients for more than four cycles of therapy (approximately three months), ranging from 79-386 days.

Vincent O'Neill, M.D., Mirna's Chief Medical Officer commented, "We're pleased to report our clinical progress to date with the first microRNA candidate for cancer. MRX34 is a promising, first-in-class therapeutic candidate with potential as a single agent and in combination with targeted and immune therapies. With the safety profile and recommended dose established, we look forward to advancing the development of MRX34 into Phase 2 later in 2016."

The presentation may be accessed from the Events & Presentations section of the Company's website.

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 plans for MRX34 development; our preclinical and clinical activity and results of our clinical development program; and the potential efficacy of MRX34, including as a combination therapy. 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 Annual Report on Form 10-K filed with the SEC on March 30, 2016.

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