November 9, 2015

Mirna Therapeutics Announces Presentation of Interim Data from Ongoing Phase I Clinical Trial of MRX34, First-in-Class microRNA-34 mimic, in Patients with Advanced Solid Tumors

Data presented at AACR-NCI-EORTC International Conference

AUSTIN, Texas--(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 interim results from its ongoing Phase 1 clinical trial of MRX34, the Company's lead therapeutic product candidate.[1] The poster presentation at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in Boston, MA, reported interim Phase 1 clinical results for MRX34 from 75 patients with advanced solid tumors. MRX34 is a double-stranded "mimic" of the naturally occurring tumor suppressor microRNA (miRNA) miR-34, encapsulated in the SMARTICLES® liposomal delivery formulation.

Interim safety, efficacy and biomarker data from the multicenter, open-label Phase 1 clinical trial in solid tumor patients show that MRX34 has a safety profile manageable with standard interventions or tests used by oncologists, and demonstrate the therapeutic potential of miR-34 replacement therapy. Additionally, dose-dependent effects on miR-34 target genes in patients' white blood cells have been observed. As of August 13, 2015, two patients with advanced, metastatic Stage IV cancer have achieved clinical responses after treatment with MRX34: one patient with primary liver cancer (hepatocellular carcinoma, HCC) metastatic to the lung, and one patient with acral melanoma, metastatic to lymph nodes, showed more than 30 percent tumor shrinkage (confirmed partial responses).

The findings were presented on Sunday, November 8, in a poster presentation entitled "Safety, tolerability, and clinical activity of MRX34, the first-in-class liposomal miR-34 mimic, in patients with advanced solid tumors" by primary author Muhammad Shaalan Beg, M.D., Assistant Professor, Internal Medicine, University of Texas Southwestern Medical Center.

"These results further confirm our excitement about the MRX34 program, demonstrating early, promising therapeutic potential of microRNA replacement therapy in patients with advanced Stage IV cancer," commented Paul Lammers, M.D., President and Chief Executive Officer of Mirna. "We look forward to the expansion phase of this trial in which we expect to enroll more patients with these as well as other cancer types."

About the Phase 1 Trial

The primary objectives of the Phase 1 MRX34 study, being conducted at leading cancer research centers in the U.S. and Korea, are to determine the maximum tolerated dose and the recommended Phase 2 dose for future clinical trials. Secondary objectives include assessments of MRX34 safety, tolerability and pharmacokinetic profiles, as well as biological and/or clinical activity. The study design consisted of an initial dose-escalation phase, where MRX34 was administered intravenously (IV) to patients in one of two dosing schedules, either twice a week for three weeks with one week off, during 28-day cycles, or daily for five days with two weeks off, in 21-day cycles, until disease progression or intolerance. The expansion phase of the trial will enroll approximately 100 additional patients in multiple specific cancer types. Enrollment in the expansion phase is expected to be complete by the end of 2016.

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 expectations for the therapeutic potential of microRNA replacement therapy in patients with advanced Stage IV cancer and our expectations regarding the expansion phase of our Phase 1 clinical trial of MRX34. 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 final prospectus filed with the U.S. Securities and Exchange Commission on October 1, 2015.

[1]Beg MS, Brenner A, Sachdev J, Ejadi S, Borad M, Kang Y-K, Lim HY, Tae-You Kim T-Y, Bader AG, Stoudemire J, Smith SC, Kim S, Hong D. Safety, tolerability, and clinical activity of MRX34, the first-in-class liposomal miR-34 mimic, in patients with advanced solid tumors. AACR-NCI-EORTC Int Conf Molec Targets Cancer Ther. Nov 8, 2015. Clinical Trials Session C, Abstr #C43.

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