



**Mirna Therapeutics Presents Interim Phase 1 Data
on First-in-Class microRNA-34 mimic, MRX34, at the 26th EORTC-NCI-AACR
Symposium on Molecular Targets and Cancer Therapeutics**

--MRX34 found to have manageable safety profile in two different treatment regimens--

Austin, Texas – November 19, 2014 – Mirna Therapeutics (Mirna), a private, clinical-stage biopharmaceutical company focused on the development of microRNA-based oncology therapeutics, today announced interim safety and preliminary efficacy data from a multicenter, open-label Phase 1 clinical trial of MRX34. The data show that MRX34 has a manageable safety profile in patients with advanced primary liver cancer (hepatocellular carcinoma), other solid tumors with liver metastasis, and hematological malignancies. A maximum tolerated dose (MTD) was established at 110 mg/m² for MRX34 administered twice weekly for three weeks followed by one week off. Dose escalation is on-going for a second dosing regimen wherein MRX34 is administered daily for five consecutive days followed by two weeks off. While this Phase 1 study is intended to investigate safety, tolerability, pharmacokinetics, and dosing regimens, treatment with MRX34 has provided early signals of clinical activity in advanced cancer patients with primary liver, neuroendocrine, colorectal and small cell lung cancers, as well as diffuse large B-cell lymphoma.

The findings were presented by Muhammad Shaalan Beg, M.D., Assistant Professor of Internal Medicine and co-leader of the gastro-intestinal oncology group at University of Texas Southwestern Harold C. Simmons Cancer Center in Dallas, Texas, at the 26th EORTC-NCI-AACR Symposium on Molecular Targets and Cancer Therapeutics hosted by the European Organisation for Research and Treatment of Cancer (EORTC), the National Cancer Institute (NCI), and the American Association for Cancer (AACR) taking place in Barcelona, Spain from November 18-21, 2014.

“As the first microRNA mimic to enter clinical development, the data reported today represent another step forward for MRX34, our lead product candidate,” said Paul Lammers, M.D., President and Chief Executive Officer of Mirna. “We are very excited to make progress toward establishing a recommended dose and dose regimen for the continued clinical development of MRX34.”

About the Phase 1 Trial

The Phase 1 MRX34 study design consists of an initial dose-escalation phase, followed by an expansion phase in one or more focused cancer types. In the study, MRX34 is 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. Dose escalation in the daily times five dosing schedule is currently ongoing. The primary objectives of the clinical trial are to establish the maximum tolerated dose and the recommended Phase 2 dose for future clinical trials. The

secondary objectives are to assess the safety, tolerability and pharmacokinetic profile of MRX34 after IV dosing as well as to assess pharmacodynamics and clinical activity of MRX34. Clinical activity is assessed by tumor response using RECIST, modified RECIST (primary liver cancer), or other cancer-specific criteria (hematologic malignancies) and evaluated by Computed Tomography/Magnetic Resonance Imaging (CT/MRI), Positron Emission Tomography/Computed Tomography (PET/CT), or other standard methods every six to eight weeks. The study is being conducted at leading cancer research centers in the U.S. and Korea.

Data from 52 patients are included in the interim analysis, which showed that treatment emergent adverse events primarily consisted of infusion reactions such as fever, chills, nausea, vomiting, back and flank pain. The addition of dexamethasone, a corticosteroid, as premedication was found to ameliorate infusion reactions. Other treatment emergent adverse events included fatigue, diarrhea, headache, dehydration, elevation of liver enzymes, decreased albumin, hyponatremia, lymphopenia, thrombocytopenia, and neutropenia.

About MRX34

MRX34 is a double-stranded microRNA “mimic” of the naturally occurring tumor suppressor miR-34, which inhibits cell cycle progression and induces cancer cell death. Mirna filed its first Investigational New Drug (IND) Application with the U.S. Food and Drug Administration (FDA) for MRX34 in early 2013 and initiated the ongoing Phase 1 clinical trial in April 2013, making MRX34 the first microRNA replacement therapy product candidate to enter a clinical trial in cancer. MRX34 is delivered using the SMARTICLES[®] liposomal delivery formulation, in-licensed from Marina Biotech.

About Mirna Therapeutics, Inc.

Mirna Therapeutics, Inc. (Mirna) is a clinical-stage biopharmaceutical company developing a broad pipeline of leading microRNA-based oncology therapeutics. Mirna’s lead program, MRX34, a first in class cancer compound, is the first microRNA mimic drug candidate to advance into clinical testing, and is now being studied in a Phase 1 trial in patients with liver cancers and hematological malignancies such as lymphoma and leukemia. Mirna's patent portfolio relating to its proprietary microRNA mimics technology consists of nine issued U.S. patents that include cancer and non-cancer therapeutic use claims related to 15 tumor suppressor microRNAs and more than 100 U.S. and foreign pending patent applications that it either owns or in-licenses from third parties. The company, founded in 2007 and located in Austin, TX, has received significant funding from New Enterprise Associates, Pfizer Venture Investments, Sofinnova Ventures and other private investors. Mirna is also funded by the State of Texas, both through the State’s Emerging Technology Fund, and from CPRIT.

For more information, visit www.mirnarx.com.

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