Mirna Therapeutics Presents Interim Phase 1 Data
on First-in-Class, First-in-Clinic microRNA-34 mimic, MRX34,
at the 2015 Annual Meeting of the American Association for Cancer Research

--MRX34 demonstrates dose dependent repression of multiple key oncogenes in white blood cells from patients with multiple tumor types--
--miR-34 directly represses the checkpoint signaling molecule PD-L1--

Austin, Texas – April 21, 2015 – Mirna Therapeutics (Mirna), a private, clinical-stage biopharmaceutical and immuno-oncology company focused on the development of microRNA-based cancer therapeutics, today announced two data presentations on MRX34, the company's lead therapeutic product candidate, at the Annual Meeting of the American Association for Cancer Research (AACR), taking place in Philadelphia, Pennsylvania from April 18-22, 2015. 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 and preliminary biomarker data from a multicenter, open-label Phase 1 clinical trial show that MRX34 continues to have a manageable safety profile in patients with advanced primary liver cancer (hepatocellular carcinoma), other solid tumors with or without liver metastasis, and hematological malignancies. A molecular analysis of white blood cells from patients treated with MRX34 in the study shows a dose dependent repression of several key oncogenes that have previously been identified as direct miR-34 targets including FOXP1, BCL2, HDAC1 and CTNNB1. These data suggest delivery of miR-34 into human white blood cells and engagement of several biological targets of the miRNA.

The findings will be presented on April 21, by David S. Hong, M.D. in an oral presentation entitled "Preclinical and clinical development of microRNA-34 mimic, MRX34, for treatment of liver cancer." Dr. Hong serves as Deputy Chair and Associate Professor, Department of Investigational Cancer Therapeutics, Division of Cancer Medicine, at The University of Texas MD Anderson Cancer Center, Houston, Texas.

A second presentation includes new *in vivo* data from a mouse model of non-small cell lung cancer (NSCLC), showing that miR-34 directly represses the checkpoint signaling molecule PD-L1 (programmed death ligand 1) and that MRX34 treatment leads to an increase in active tumor-infiltrating immune cells (CD8+) and a decrease in exhausted tumor-infiltrating immune cells (CD8+PD1+). These data were generated in collaboration with James W. Welsh, M.D., Associate Professor, Department of Radiation Oncology, Division of Radiation Oncology, The University of Texas MD Anderson Cancer Center, Houston, Texas, and were presented on April 20, in an oral presentation entitled "p53 regulation of PDL1 is mediated through miR-34a".

"We are very excited about the continued progress of our MRX34 program. As the first microRNA mimic in clinical development, the data reported this week represent substantial

progress in demonstrating our ability to deliver miR-34 in a dose-dependent fashion into cells in patients and engagement of its biological targets," said Paul Lammers, M.D., President and Chief Executive Officer of Mirna. "Furthermore, the newly elucidated role of miR-34 in tumor immune evasion pathways opens up additional opportunities, and further supports the broad potential of microRNA Replacement Therapy,"

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 establish the maximum tolerated dose and the recommended Phase 2 dose for future clinical trials. The study design consists of an initial dose-escalation phase, followed by an expansion phase in multiple specific 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.

Data from 23 patients with hepatocellular carcinoma are included in the interim analysis, which show that treatment emergent adverse events primarily consist of infusion reactions such as fever, chills, back pain, nausea, vomiting, and diarrhea. The addition of dexamethasone, a corticosteroid, as premedication has been found to ameliorate infusion reactions. Other common treatment-emergent adverse events include fatigue, anorexia, headache, elevation of liver enzymes, decreased albumin, hyponatremia, hyperglycemia, lymphopenia, thrombocytopenia, and neutropenia. A maximum tolerated dose (MTD) has been established in the twice weekly regimen, whereas dose escalation continues in the daily times five dosing schedule.

The secondary objectives of the study 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. The Phase 1 clinical trial is expected to enroll approximately 120 patients and final results are expected in late 2015.

About Mirna Therapeutics, Inc.

Mirna Therapeutics, Inc. (Mirna) is a clinical-stage biopharmaceutical and immuno-oncology 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 miRNA mimic drug candidate to advance into clinical testing. Mirna's patent portfolio relating to its proprietary microRNA mimics technology consists of twelve issued U.S. patents that include cancer and non-cancer therapeutic use claims related to 17 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 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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