



## **Mirna Therapeutics Announces First Patient Enrolled in Hematological Malignancy Cohort of its Phase 1 Clinical Trial**

Austin, Texas – June 10, 2014 – Mirna Therapeutics, Inc. (Mirna), a clinical-stage biopharmaceutical company pioneering microRNA-based Replacement Therapy to treat cancer, today announced the enrollment of the first patient in the hematological malignancy cohort of its ongoing Phase 1 clinical trial of MRX34, the company’s lead product candidate and first microRNA mimic in human clinical trials in oncology.

“We believe microRNA therapeutics hold significant promise in the fight against cancer and we are excited to expand the ongoing clinical study of MRX34 in liver-based cancers, to now also include patients with hematological malignancies,” said Jorge Cortes, M.D., Deputy Department Chair, Department of Leukemia, Division of Cancer Medicine at MD Anderson Cancer Center and a Principal Investigator of the study.

The multicenter, open-label Phase 1 clinical trial of MRX34 was initiated in April 2013 and is currently enrolling patients with unresectable primary liver cancer or solid cancers with liver involvement. The trial is now enrolling a separate cohort of patients with hematological malignancies, which are cancers that affect blood, bone marrow and lymph nodes and may include non-Hodgkin’s lymphoma, acute myelogenous leukemia, acute and chronic lymphocytic leukemia, chronic myelogenous leukemia in accelerated or blast phase, multiple myeloma or myelodysplastic syndrome.

An interim analysis of safety data from the first 26 patients was recently presented at the American Association of Clinical Research (AACR) annual meeting. The interim data showed that the most common adverse events associated with MRX34 in the patients studied have been manageable with standard interventions used by oncologists, with one incident of a dose-limiting toxicity observed. The maximum tolerated dose had not been reached and additional patients are being enrolled into the study.

### **About the Phase 1 Trial**

The Phase 1 clinical trial design consists of an initial dose-escalation phase, followed by an expansion phase after the recommended Phase 2 dose has been identified. In the liver-based cancer cohort, which aims to enroll approximately 48 patients, MRX34 is administered intravenously twice a week for three weeks with one week off, during 28-day cycles, until disease progression or intolerance. In the heme malignancy cohort, which aims to enroll approximately 15 patients, patients will be treated continuously for five days with MRX34, followed by two weeks off, during 21-day cycles, until disease progression or intolerance. The primary objectives of both clinical trial cohorts 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 intravenous dosing as well as to assess biological and clinical activity.

#### **About MRX34**

MRX34, a first in class cancer therapy, was designed to deliver a mimic of the naturally occurring microRNA tumor suppressor, miR-34, which is under expressed in tumors of patients with a wide variety of cancers, including cervical cancer, ovarian cancer, glioblastoma, hepatocellular carcinoma (liver cancer), colon cancer and non-small cell lung cancer, and in cancer stem cells. The miR-34 mimic is encapsulated using an innovative liposomal formulation called SMARTICLES®, which Mirna has licensed from Marina Biotech. 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.

This clinical trial is supported in part by a commercialization grant from the Cancer Prevention and Research Institute of Texas (CPRIT).

Additional information on the Phase 1 clinical trial and enrollment can be found at [clinicaltrials.gov](http://clinicaltrials.gov) (<http://clinicaltrials.gov/ct2/show/NCT01829971>).

#### **About Mirna Therapeutics, Inc.**

Mirna Therapeutics, Inc. (Mirna) is a clinical-stage biopharmaceutical company developing a broad pipeline of leading microRNA-based oncology therapeutics. In 2013, Mirna initiated a Phase 1 clinical trial of MRX34, a first in class cancer compound, for the treatment of liver-based cancers, making it the first microRNA mimic drug candidate to advance into clinical testing in cancer patients. 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](http://www.mirnarx.com).

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