



Mirna Therapeutics Presents New Data on the Development of miRNA-based Therapeutic Candidates for Hepatocellular Carcinoma

- Tumor-suppressing miRNAs significantly inhibited tumor growth in mice -

AUSTIN, TX – November 15, 2011. Mirna Therapeutics, Inc., a biotechnology company pioneering microRNA replacement therapy for cancer, announced the presentation today of new preclinical data on the development of a miRNA-based therapeutic candidate for primary liver cancer at the *AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics* in San Francisco, CA. The data were simultaneously presented at the *2011 CPRIT Innovations in Cancer Prevention and Research Conference* in Austin, TX.

In a poster entitled “The Development of a miRNA-based Therapeutic Candidate for Hepatocellular Carcinoma,” Mirna scientists showed that mimics of five tumor suppressor miRNAs, including miR-34 and let-7, all significantly inhibited the growth of liver tumors compared to animals treated with formulated negative control miRNAs. The five miRNA mimics were complexed with a lipid nanoparticle delivery formulation and injected into NOD/SCID mice with orthotopically grown Hep3B human liver cancer xenografts.

Preliminary studies indicate that the formulated miRNAs are neither toxic nor immunostimulatory. The formulated miRNAs are currently being evaluated for therapeutic activity in mouse models of other cancers and a development program has been initiated in anticipation of creating candidates for clinical cancer therapy.

The outstanding efficacy produced by miRNA replacement therapy results from the capacities of miRNAs to regulate multiple cancer-related genes and pathways. As a key part of the p53 tumor suppressor pathway, miR-34, for instance, regulates the expression of *MET*, *MYC*, *RRAS*, *Notch*, *WNT*, and other oncogenes.

“These data contribute to the mounting evidence that miRNA replacement therapy holds significant promise as a new approach to the treatment of cancer, and adds to our previous work showing that there are multiple potential delivery approaches that can be successfully employed to deliver Mirna’s proprietary microRNAs to orthotopically grown tumors,” said Dr. Paul Lammers, President and Chief Executive Officer of Mirna Therapeutics.

About microRNAs

miRNAs are approximately 20-25 nucleotides long and affect gene expression by interacting with messenger RNAs. Unlike siRNAs, miRNAs are encoded in the human genome and are used as natural regulators of global gene expression. More than 1,400 miRNAs are encoded in the human genome and comprise approximately 2% of all mammalian genes. Since each miRNA appears to regulate the expression of tens to hundreds of different genes, miRNAs can function as “master-switches,” efficiently regulating and coordinating multiple cellular pathways and processes. By coordinating the expression of multiple genes, miRNAs are responsible for guiding proper embryonic development, immunity, inflammation, as well as cellular growth and proliferation. Misregulation of miRNAs appears to play a fundamental role in the occurrence, growth and dissemination of many cancers, and replacement of down regulated miRNAs in tumor cells results in a positive therapeutic response.

About Mirna Therapeutics

Mirna Therapeutics is a biotechnology company focused on the development and commercialization of microRNA (miRNA) therapeutics. The Company has a substantial intellectual property portfolio on the therapeutic use of miRNAs developed by its own scientists as well as in-licensed from other institutions. Mirna’s IP portfolio contains >300 miRNAs with applications in oncology and other diseases. Oncology-directed miRNAs include those that are key tumor suppressors in cancer, such as *miR-34* and *let-7* that have proven to block tumor growth in a number of different pre-clinical animal studies. The Company, founded in 2007, is located in Austin, Texas, and has received significant funding from the State of Texas, both through the State’s Emerging Technology Fund and from the Cancer Prevention and Research Institute of Texas (CPRIT). Mirna Therapeutics is the recipient of a \$10.3 million commercialization award from CPRIT. For more information, visit www.mirnarx.com



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