



Tiny RNA Has Big Impact On Lung Cancer Tumors

New Haven, Connecticut and Austin, Texas – December 7, 2009. Researchers from Yale University and Mirna Therapeutics, Inc., reversed the growth of lung tumors in mice using a naturally occurring tumor suppressor microRNA. The study reveals that a tiny bit of RNA may one day play a big role in cancer treatment, and provides hope for future patients battling one of the most prevalent and difficult to treat cancers.

“This is the first time anybody has shown a positive effect of microRNAs in shrinking lung cancer,” said Frank Slack, Ph.D., co-senior author of the paper, researcher at the Yale Cancer Center and professor of molecular, cellular & developmental biology.

Paul Lammers, President & CEO of Mirna Therapeutics said, “This data further confirms the importance microRNAs play in cancer and the opportunity that microRNA replacement therapy represents. By working with researchers such as Dr. Slack, Mirna is fulfilling its mission to become one of the leading microRNA therapeutics companies.”

The tumors in mice with non-small cell lung cancer shrank after the Yale team delivered an intranasal dose containing a type of micro-RNA called *let-7*, the authors reported in the Dec. 7 issue of the journal *Oncogene*. MicroRNAs are small bits of genetic material most often associated with transmission of information encoded in DNA. However in the past decade microRNAs have been shown to play crucial roles in gene regulation and/or gene silencing.

The Yale team also found that mice without *let-7* developed cancer, supporting their hypothesis that the microRNA acts as a tumor suppressor. The tumors in mice that received *let-7* were not eliminated, but reduced by 66 percent, the study showed. The team is currently studying whether *let-7* therapy in combination with chemotherapy and radiation can induce full remission.

Slack noted *let-7* is absent in many cancers and acts upon a gene known to play a role in about a quarter of all human cancers. “We hope it will be valuable in the treatment of many other forms of cancer,” he said.

The research was conducted as part of a collaboration between Yale and Mirna Therapeutics Inc, a biotechnology company in Austin, Texas. Joanne B. Weidhaas, MD/Ph.D. of Yale and Andreas G. Bader, Ph.D. of Mirna were co-senior authors of the paper. Other Yale authors on the paper are first author Phong Trang, Pedro P. Medina and Robert Homer; other Mirna authors are Jason F. Wiggins, Lynnsie Ruffino, Kevin Kelnar, Michael Omotola and David Brown, Ph.D.

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About microRNA

MicroRNAs are approximately 21 nucleotide long RNA molecules that affect gene expression by interacting with messenger RNAs. Unlike siRNAs, miRNAs are encoded in the human genome and are natural regulators of global gene expression. More than 700 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. Mis-regulation of miRNAs appears to play a fundamental role in many cancers and replacement of down regulated miRNAs in tumor cells by “MicroRNA Replacement Therapy” may result in a positive therapeutic response.

About Mirna Therapeutics

Mirna Therapeutics is a biotechnology company focused on the development and commercialization of microRNA (miRNA) therapeutics. Mirna has a substantial body of pending intellectual property around miRNAs developed by its own scientists as well as in-licensed from other institutions. Mirna scientists, while at Ambion, filed two very broad patent applications in 2004 naming more than 200 miRNAs and their functional roles in a number of different disease states. Mirna owns more than thirty pending patent applications as well as exclusive

rights to pending patents submitted by Yale University. Mirna's IP portfolio contains numerous miRNAs with applications in oncology including those that are key tumor suppressors in cancer. Among Mirna's lead candidates for therapeutic development are the tumor suppressors *miR-34* and *let-7* that have proven to block tumor growth in a number of different pre-clinical animal studies (Johnson et al., Cell, 2005; Esquela-Kerscher et al., Cell Cycle, 2008; Trang et al., Oncogene, 2009). More information is available at the Company's website: www.mirnarx.com.

About Yale University

Yale is a leading university dedicated to promoting health worldwide through advanced biomedical research, graduate-level education in the life sciences and health professions, and excellence in patient care. For further information, please visit www.yale.edu.

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