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**miRagen Announces Discovery of Key microRNA Implicated in Lou Gehrig's Disease  
Findings Could Lead to Novel Therapeutic Intervention Strategies for Neuromuscular Disorders  
Data published in latest issue of Science**

**BOULDER, Colo., December 10, 2009** – miRagen Therapeutics, Inc., a biopharmaceutical company focused on improving patients' lives by developing innovative microRNA-based therapeutics for cardiovascular and muscle disease, today announced the publication of data demonstrating that microRNA-206 (miR-206) plays a crucial role in the progression of amyotrophic lateral sclerosis (ALS or Lou Gehrig's disease) and in neuromuscular synaptic regeneration in mice. The findings, published in the December 11 issue of the journal *Science*, reveal miR-206 as a potential target for the development of therapeutic agents for the treatment of neuromuscular disease. The study was conducted by researchers led by Eric N. Olson, Ph.D., at the University of Texas Southwestern Medical Center. miRagen is the exclusive licensee of intellectual property (IP) rights related to this discovery, which are part of the Company's extensive estate of enabled microRNA IP.

"This is a breakthrough with significant implications for human health," said William S. Marshall, Ph.D., President and CEO of miRagen Therapeutics, Inc. "Currently there are no therapies available to ALS patients that will reverse or delay the onset of muscle atrophy associated with this debilitating disease. Uncovering the essential role of miR-206 in neuromuscular maintenance brings us one step closer to the day when physicians will be able to offer hope to those suffering from ALS and other neuromuscular diseases."

"We are, quite frankly, excited by these findings," said Dr. Olson, Chairman and Professor of Molecular Biology at the University of Texas Southwestern Medical Center and miRagen's Chief Scientific Advisor. "One of the hallmarks of ALS and other similarly degenerative muscle diseases is the inability of the neuromuscular synapse to transmit the impulse that leads to muscle contraction. Our data show that miR-206 plays a central regulatory role in this process. We view this as an extremely promising target for therapeutic intervention."

**About microRNAs**

MicroRNAs have emerged as an important class of small RNAs encoded in the genome. They act to control the expression of sets of genes and entire pathways and are thus thought of as master regulators of gene expression. Recent studies have demonstrated that microRNAs are responsible for many diseases. Because they are single molecular entities that dictate the expression of fundamental regulatory pathways, microRNAs represent potential drug targets of unprecedented power.



### **About miRagen Therapeutics**

Cardiovascular disease is the leading cause of death globally. Astonishingly, in the United States, every 37 seconds a person dies from cardiovascular disease. For survivors, life changes dramatically after an adverse heart event. Because of the significant toll that cardiovascular and muscle disease takes on patients, miRagen Therapeutics, Inc., was founded in 2007 to develop innovative microRNA-based therapeutics for cardiovascular and muscle disease. Only recently discovered, microRNAs are short, single-stranded RNA molecules encoded in the genome that regulate gene expression and play a vital role in influencing cardiovascular and muscle disease. Principally funded through venture capital investments, miRagen combines world recognized leadership in cardiovascular medicine with unprecedented in-house expertise in microRNA biology and chemistry. For more information, please visit [www.miRagenTherapeutics.com](http://www.miRagenTherapeutics.com).

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