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VIRxSYS Publishes New Study on RNA Therapy
New RNA therapy reversed hemophilia in mice

Gaithersburg, MD - (April 6, 2009) - VIRxSYS Corporation announced today the publication of a new research article, “*Trans*-splicing into Highly Abundant Albumin Transcripts for Production of Therapeutic Proteins *In Vivo*,” in the journal *Molecular Therapy*. The study describes a novel RNA therapy technique in which an RNA molecule that is inserted into the albumin gene “hijacks” the albumin, causing it to produce the newly added gene’s proteins instead of its own. Using this strategy, the researchers were able to produce three different therapeutic proteins in animals. With one of these, Factor VIII, they showed they could correct hemophilia A in mice. The researchers believe their technology can be used to produce therapeutic proteins in humans.

“Two of the RNA molecules that were spliced into albumin are normally produced in the liver. These are the apoA-I, which produces the principal protein in good cholesterol, and Factor VIII, which makes the protein missing in patients with hemophilia A. We instructed the liver to make more apoA-I protein than it normally does, and in separate experiments we instructed the liver to make the correct form of Factor VIII instead of a mutated form. We also added an RNA molecule that makes a monoclonal antibody that is not normally produced in liver,” said VIRxSYS Executive Vice President of Scientific and Clinical Affairs, Gerard J. McGarrity, PhD. “The work is at an early stage, but we are definitely teaching old genes some new tricks.”

Albumin is the most abundantly expressed gene in liver. Results showed that each of the three gene sequences spliced into the albumin gene sequence was expressed in high concentrations by liver cells in laboratory mice. The specific approach that is used ensures that added RNA was only expressed in liver cells, and not in other cells, which could have triggered an immune response. The technology used to perform the albumin splicing process is owned by VIRxSYS and is known as spliceosomal mediated RNA *trans*-splicing, or SMaRT™. VIRxSYS now has 39 publications in top tier journals showing the effectiveness of SMaRT™ in a broad variety of applications. The company expects to begin testing this technology in human trials in the second half of 2010.

The study was headed by Madaiah Puttaraju, Ph.D., of VIRxSYS. The full citation for the study is: Wang J, Mansfield SG, Cote CA, Jiang PD, Weng K, Amar MJ, Brewer BH Jr, Remaley AT, McGarrity GJ, Garcia-Blanco MA, Puttaraju M. *Trans*-splicing into highly abundant albumin transcripts for production of therapeutic proteins in vivo. *Mol Ther*. 2009; 17(2):343-51; doi:10.1038/mt.2008.260. Drs. Amar, Brewer and Remaley were collaborators at the National Heart, Lung and Blood Institute (NHLBI) of the National Institutes of Health. Part of these studies was performed under a cooperative research and development agreement (CRADA) between VIRxSYS and the NHLBI.

About VIRxSYS

Founded in 1998, VIRxSYS is a private biotechnology company that focuses on the development of a novel lentiviral gene delivery platform technology for the treatment of serious diseases. The Company has exclusively licensed its patented, proprietary lentiviral technology platform from The Johns Hopkins University (JHU) in Baltimore, Maryland where the original research was conducted. The Company obtained its SMaRT™ technology from Intronn Inc. More information regarding VIRxSYS can be found at www.virxsys.com.

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