



## **REGENX BioSciences Highlights Gene Therapy Advances in the Prevention and Treatment of HIV and Hemophilia B using NAV™ rAAV8 Vectors**

*Landmark Human Clinical Study Demonstrates Durable Correction of Inherited Bleeding Disorder using NAV rAAV8 Vectors Encoding Gene for Missing Clotting Factor*

WASHINGTON, DC Dec. 14, 2011 -- REGENX BioSciences announced that two recently published studies using REGENX NAV technology provide data demonstrating (1) correction of an inherited bleeding disorder, hemophilia B, in a human clinical study using NAV rAAV8 vectors to express a crucial, missing clotting factor and (2) that NAV rAAV8 vectors encoding genes for anti-HIV neutralizing antibodies can generate sufficient levels of antibodies in the circulation of mice to protect them against HIV infection. The studies highlight the safety and effectiveness of NAV™ technology as a platform for the development of therapeutics for the treatment of a range of severe diseases.

“Together, these studies demonstrate the versatility of the NAV technology in preventing and treating disease,” said Kenneth Mills, REGENX president and chief executive officer. “Using NAV rAAV8 vectors is the basis of several of our development programs to treat genetic forms of ocular and metabolic disease. This recent data supports the potential of NAV rAAV8 vectors to yield a pipeline of treatments for a wide range of diseases and to address significant unmet medical needs.”

### **NAV rAAV8 in Clinical Study for Treatment of Hemophilia B**

[“Adenovirus-Associated Virus Vector-Mediated Gene Transfer in Hemophilia B”](#), published online by *The New England Journal of Medicine* on December 10, 2011, reported on a combined Phase 1/2 clinical trial involving NAV rAAV8-mediated gene transfer in patients with hemophilia B. In this trial, six hemophilia B patients received a single intravenous injection of NAV rAAV8 vectors encoding a normal copy of the defective gene, Factor IX. All patients are expressing enough Factor IX to convert their phenotypes from severe to mild-moderate. Four out of six patients have been able to discontinue use of prophylactic, recombinant Factor IX and have not experienced spontaneous bleeding since receiving the therapy. The other two patients have been able to extend the time between prophylactic treatments. Most importantly, the therapeutic was well-tolerated.

“This study is an important milestone. It is the first success of in vivo therapeutic gene delivery in treating bleeding disorders and embodies the first clinical use of NAV rAAV8 vectors,” said Karen Kozarsky, Ph.D., vice president research & development at REGENX. “The success observed using NAV rAAV8 vectors in this landmark study for hemophilia B validates our approach using NAV rAAV8 vectors in the development of other treatments for familial hypercholesterolemia (FH) and Hurler syndrome (MPS I). These results illustrate the therapeutic potential of NAV technology as a true platform for treating a range of other gene and protein deficiencies.”

The Phase 1/2 clinical trial was carried out by investigators from University College London and St. Jude Children's Research Hospital and the results were also reported at the 2011 American Society of Hematology Annual Meeting.

### **NAV rAAV8 in Therapeutic Research for Protection from HIV Infection**

["Antibody-based protection against HIV infection by vectored immunoprophylaxis,"](#) published online in *Nature* on November 30, 2011, highlights the potential of a safe, efficient, and long-lasting delivery of antibodies for immunization against acquired infection. The work was carried out in the laboratory of Nobel prize-winning scientist Dr. David Baltimore at California Institute of Technology.

Dr. Baltimore and his colleagues reported that NAV rAAV8 vectors encoding genes for anti-HIV neutralizing antibodies, when injected into the muscle of mice, can direct the synthesis and secretion of therapeutic levels of those antibodies into the circulation. They further demonstrated that the antibodies are capable of protecting the experimental animals from HIV challenge. This approach is designated "passive immunization", in which an individual is given high levels of an antibody that has already been demonstrated to be capable of neutralizing HIV to prevent infection. To avoid repetitive delivery of purified antibodies over a lifetime, researchers adopted gene delivery with NAV rAAV8 vectors involving a one-time injection of the vector, which directs cells to synthesize and secrete the antibody long-term.

"This work demonstrates a potential new avenue for protection from infection, particularly in diseases where traditional vaccine approaches have been ineffective," said Dr. Kozarsky.

#### *About REGENX BioSciences*

REGENX BioSciences is leading the effort to translate promising gene delivery applications into a pipeline of next generation personalized therapies for a range of severe diseases with serious unmet needs. We believe that the **NAV<sup>TM</sup>** technology to which we have exclusive rights represents the potential promise of curing the root cause of disease rather than the symptoms, and we are committed to establishing best in class standards for our **NAV** vectors. Our intent is to initially develop treatments for a number of rare, genetic diseases including hypercholesterolemias, the mucopolysaccharidoses, and retinitis pigmentosa and to ensure continuing access to our **NAV** technology through innovative partnerships, license opportunities, as well as the expansion of our growing team of global collaborators. REGENX holds exclusive rights to a portfolio of over 100 patents and patent applications pertaining to its **NAV** technology and related applications. Visit [www.regenxbio.com](http://www.regenxbio.com) to learn more.

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