ReGenX BioSciences Supports Rare Disease Day: Highlights Gene Therapy Advances

(Washington DC February 28)-----ReGenX BioSciences will be joining the National Organization for Rare Disorders (NORD) and others around the world in observing World Rare Disease Day on February 28, 2011. On this day, millions of patients and their families will share their stories to focus a spotlight on rare diseases as an important global public health concern.

"There are nearly 30 million Americans—and millions more around the world—affected by rare diseases," said Peter L. Saltonstall, president and CEO of NORD. "Everyone knows someone with a rare disease. But, while many of these diseases are serious and lifelong, most have no treatment and many are not even being studied by researchers. This leaves patients and families without hope for a better future."

A rare disease is one that affects fewer than 200,000 Americans. There are nearly 7,000 such diseases affecting nearly 30 million Americans.

ReGenX BioSciences is developing the next generation of personalized treatments for rare and genetic diseases where there remains significant unmet medical need. Over the past decade, the field of gene therapy has re-emerged with compelling research that is resulting in significant improvements in safety, specificity and efficient delivery of genetic material. ReGenX Biosciences has implemented these improvements to advance closer to its goal of using gene therapy to produce a therapeutic effect in a safe manner.

Through its collaboration with researchers at the University of Pennsylvania and through licenses from the University of Pennsylvania and GlaxoSmithKline, ReGenX has developed ***NAV™*** - a gene delivery technology that includes proprietary recombinant adeno-associated viral (rAAV) vectors that are being studied in investigator sponsored human clinical studies.

Some clinical research highlights with the ***NAV*** vectors include:

Batten Disease

Late Infantile Neuronal Ceroid Lipofuscinosis (LINCL) is a form of Batten Disease, a rare, inherited neurodegenerative disease affecting children, which is fatal by 8 – 12 years of age. It is characterized by cognitive impairment, progressive vision loss, seizures, and deteriorating motor function. It is caused by mutations in the CLN2 gene which encodes tripeptidyl-peptidase I (TPP-1). Gene therapy for LINCL involves using ***NAV*** rAAVrh.10 injection to deliver a normal copy of CLN2 into the brain, which is anticipated to slow or stop the neuronal degeneration.

Researchers at Weill Cornell Medical College in a Phase I trial have reported treating one patient, a seven-year-old child, with ***NAV*** vector rAAVrh.10. No serious adverse events have been observed. Investigators plan to enroll 16 patients in this trial.

Hemophilia

Hemophilia is a genetic bleeding disorder that prevents the blood from clotting normally. The main symptom is uncontrolled, often spontaneous bleeding. Internal bleeding into the joints can result in pain, swelling and, if left untreated, can cause permanent damage.

Researchers at St. Jude's Children's Research Hospital, studying Hemophilia B patients, reported promising safety results from a Phase I/II gene therapy trial using ***NAV*** rAAV8 at a recent American Society of Hematology annual meeting.

The St. Jude's investigators reported no toxicity in four patients. In one patient, levels of factor IX protein increased from less than 1% to more than 2% of normal and the patient has not required treatment with clotting factors for ten months.

Rare Disease Day 2011 activities in the U.S. will include creating an online library of two-minute videos about specific rare diseases and how they affect patients' daily lives. Also, patients across the nation will help NORD create a database of physician experts. Patients will share

their personal stories through a survey hosted by NORD and the Pew Research Center, and there will be a drive to enlist support for a new Rare and Neglected Diseases Congressional Caucus.

For more information about Rare Disease Day activities in the U.S., go to www.rarediseaseday.us. For information about global activities, go to www.rarediseaseday.org. Global Gene Projects

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