REGENX BioSciences Supports Rare Disease Day: Highlights Recent Advances with NAV™ Technology

Clinical Study Initiated with Novel Treatment for Sanfilippo Syndrome using NAV rAAVrh.10 Vector

Washington DC February 29, 2012 -- REGENX BioSciences joins the National Organization for Rare Disorders (NORD) and others around the world in observing Rare Disease Day on February 29. The purpose is to focus attention on the needs of patients and families affected by rare diseases.

REGENX, along with its collaborators and licensees, 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 has implemented these improvements to advance closer to its goal of using gene therapy to produce a therapeutic effect in a safe manner. REGENX NAV technology - a gene delivery technology that includes proprietary recombinant adeno-associated viral (rAAV) vectors - is being studied in several on-going human clinical studies for rare diseases.

Recent clinical research highlights with the NAV vectors include:

Sanfilippo Syndrome (MPS III) Type A

Sanfilippo syndrome is one of a family of lysosomal storage diseases called mucopolysaccharidoses (MPS). These are inherited diseases caused by a defect in one of the genes needed to break down glycosaminoglycans (GAGs) – long chain sugars. There are four main types of Sanfilippo syndrome; including Sanfilippo Type A (MPSIII A), the most severe form. MPSIII A patients suffer a defect in a gene that encodes for a protein called sulfamidase (SGSH). In this disease, the accumulation of incompletely degraded GAGs primarily affects the central nervous system, resulting in delayed development, mental retardation along with other symptoms, and is fatal in childhood. According to the National MPS Society, the incidence of MPS III (all four types combined) is estimated to be 1 in 70,000 births.

A pioneering biotechnology company based in France, LYSOGENE, has announced that it has initiated the clinical study of its experimental investigational drug, SAF-301, intended to treat Sanfilippo Syndrome Type A. SAF-301 is a novel treatment designed to deliver a normal copy of the gene for SGSH directly to the central nervous system of affected individuals. It consists of the NAV rAAVrh.10 vector, carrying genes encoding SGSH and also sulfatase modifying factor 1 (SUMF1), which enhances SGSH activity. Preclinical studies have evidenced very promising safety and efficacy profiles.

The clinical protocol approved consists on a direct injection of SAF-301 to the brain in a single neurosurgical session. The primary objective is to evaluate the clinical, radiological, biological tolerance associated with SAF-301. A secondary objective of the study is to collect data to define exploratory tests that will become evaluation criteria for further clinical phase III efficacy studies (including brain MRI; neurological and biological markers). Four patients will be included in this first study of SAF-301.

More information about the SAF-301 clinical study can be found at LYSOGENE's website, www.lysogene.com and at the NIH ClinicalTrials.gov website, www.clinicaltrials.gov.

Rare Disease Day 2012

Rare Disease Day 2012 activities in the U.S. will include a "Handprints Across America" campaign to create a gallery of photos on the Rare Disease Day website; educational materials for classroom teachers; and a nationwide blitz of patient photos, stories and videos to increase awareness of specific rare diseases and the challenges of living with a rare disease. Several special events are planned, including a scientific symposium at the National Institutes of Health (NIH) and a Rare Disease Patient Advocacy Day at the Food and Drug Administration (FDA).

"This is a global observance," said Peter L. Saltonstall, president and CEO of NORD. "Individuals and organizations around the world will all be sharing stories of how rare diseases affect their lives."

In the U.S., any disease affecting fewer than 200,000 Americans is considered rare. According to the National Institutes of Health (NIH), there are nearly 7,000 such diseases affecting nearly 30 million Americans.

"Since many of these diseases are genetic, more than half of the people who have rare diseases are children" Saltonstall said. "The problems encountered by families are enormous. It's important for these families to know they are not alone."

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.

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^{TM} technology to which we have exclusive rights represents the potential promise of curing the root cause of disease rather than the symptoms. 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 to learn more.

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