



ReGenX
BIOSCIENCES

REGENX Biosciences Receives FDA Orphan Designation for Treatment of Familial Hypercholesterolemia using NAV™ rAAV8 Vectors

WASHINGTON, DC April 10, 2012 -- REGENX BioSciences, LLC. announces that it has received orphan drug product designation from the U.S. Food and Drug Administration (FDA) for its program for the treatment homozygous familial hypercholesterolemia (HoFH) using NAV™ rAAV8 vectors.

Homozygous familial hypercholesterolemia is an ultra-rare, genetic disorder characterized by a defect in the gene coding for the receptor for low-density lipoprotein (LDLR), the major cholesterol-transport lipoprotein, resulting in elevated plasma concentration of LDL and deposition of LDL-derived cholesterol (LDL-C) in tendons and skin and in arteries. For most HoFH patients, current LDL-C lowering treatments are ineffective and life expectancy is greatly reduced due to the inevitable development of life threatening coronary artery disease. If untreated, HoFH patients usually die before the age of 30.

The REGENX program consists of a NAV rAAV8 vector expressing a normal copy of the human low-density lipoprotein receptor (hLDLR) gene. Research from REGENX academic collaborators at the University of Pennsylvania, Drs. James M. Wilson and Daniel J. Rader, has shown in a mouse model of HoFH that delivery of functional hLDLR to the liver via NAV rAAV8-mediated gene delivery results in dramatic decreases in circulating LDL-C. In collaboration with its academic collaborators, REGENX is developing a clinical program intended to support the licensure of a product for the treatment of HoFH.

“Receiving orphan drug designation from the FDA is a significant milestone for the HoFH program,” said Ken Mills, President and Chief Executive Officer of REGENX. “This designation underscores our interests in developing novel therapies for rare, genetic diseases with serious unmet medical need.”

About REGENX BioSciences, LLC

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™ 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 to learn more.

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