



**REGENX BIOSCIENCES AWARDED \$2.8 MILLION NIH GRANT FOR  
NOVEL EYE THERAPEUTIC DEVELOPMENT PROGRAM**

*Company's Gene Delivery **NAV** Technology Offers Targeted Treatment Approach for  
Retinal Degenerative Diseases*

Washington D.C. May 3, 2011 – REGENX BioSciences, a privately held biopharmaceutical company and developer of **NAV**<sup>TM</sup>, a unique gene delivery technology, announced that it received a \$2.8 million dollar grant from a highly competitive National Institutes of Health (NIH) pilot program emphasizing the acceleration of innovative technologies to improve human health. The grant, issued through the National Eye Institute (NEI), will support the advancement of REGENX's novel program using its **NAV** technology for the treatment of X-Linked Retinitis Pigmentosa (RP or X-Linked RP), a debilitating eye condition that leads to blindness and for which there is currently no treatment.

"We are pleased to have received this funding and support from NEI and recognize the critical importance of accelerating early stage development into meaningful treatments for inherited and acquired retinal diseases," said Kenneth T. Mills, President and CEO, REGENX BioSciences. "**NAV** technology is attractive because of its unique ability to safely and effectively deliver genetic material to target cells in the eye in order to achieve a specific therapeutic effect. We believe that our program holds promise for a spectrum of retinal degenerative diseases in which there is great unmet patient need."

Approximately 100,000 people in the United States and over one million people worldwide have some form of RP. X-Linked RP is one of the major causes of vision loss in children and adolescents. Most cases of X-linked RP are due to a mutation in RPGR (retinitis pigmentosa GTPase regulator), a protein primarily expressed in photoreceptor cells in the retina. Mutations in RPGR are associated with a severe form of the disease, causing early onset of disease, and a relatively fast progression. In preclinical studies across multiple animal models **NAV** technology, and in particular **NAV** rAAV8 vector, has been shown to target the photoreceptor layer with excellent efficiency and safety in order to preserve vision using gene delivery approaches. REGENX is collaborating on the grant with REGENX's scientific founder, James M. Wilson, M.D., Ph.D., of the University of Pennsylvania, School of Medicine.

*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** 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 ensure continuing access for our **NAV** technology through innovative partnerships, license opportunities and 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)

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