REGENX Biosciences and Audentes Therapeutics Enter into Exclusive License Agreement for Development of Treatments for Serious, Rare Muscle Diseases Using NAV™ Vectors

WASHINGTON & SAN FRANCISCO – July 30, 2013 – REGENX Biosciences, LLC and Audentes Therapeutics, Inc. announce that they have entered into an agreement for the development and commercialization of products to treat X-Linked Myotubular Myopathy (XLMTM) and Pompe disease using **NAV** vectors.

Under the terms of the Agreement, REGENX granted Audentes an exclusive worldwide license, with rights to sublicense, to REGENX's **NAV** rAAV8 and rAAV9 vectors for treatment of XLMTM and Pompe disease in humans. In return for these rights, REGENX receives an up-front payment, certain milestone fees and royalties on net sales of products incorporating **NAV** rAAV8 and rAAV9.

"We believe this exclusive license agreement is important to the successful development of **NAV** based gene delivery treatments for patients with XLMTM and Pompe disease," said Ken Mills, President and CEO of REGENX. "As a leader in gene therapy, we are pleased to be cooperating with the team at Audentes in its pursuit of developing innovative treatments for patients with serious, rare muscle diseases through the application of **NAV** technology. REGENX has a continued interest to provide commercial partners that evidence outstanding leadership, expertise, resources and a strong commitment to patients, such as Audentes, with access to our **NAV** technology."

"Audentes is committed to the development of new treatments for patients with XLMTM and Pompe disease using AAV gene therapy technology and we feel rAAV8 and rAAV9 are the most promising vectors to achieve this goal," said Matthew R. Patterson, President and CEO of Audentes. "We are very pleased to enter into this agreement with REGENX, which we believe offers us the best path to expeditiously develop novel therapies for patients."

About X-Linked Myotubular Myopathy (XLMTM)

X-Linked Myotubular Myopathy (XLMTM) is a rare, inherited disorder characterized by severe muscle weakness and respiratory impairment. It is caused by mutations in the *MTM1* gene, which encodes an enzyme called myotubularin. Myotubularin is thought to be involved in the development and maintenance of muscle cells. XLMTM affects approximately 1 in 50,000 newborn males worldwide.

About Pompe Disease

Pompe Disease is a rare, inherited disorder characterized by progressive muscle weakness and respiratory impairment. It is caused by mutations in a gene that encodes an enzyme called acid alpha-glucosidase (GAA), which is needed by the body to break down glycogen – a stored form of sugar used for energy. Pompe Disease affects approximately 1 in every 40,000 births.

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.

For more information regarding REGENX, please visit www.regenxbio.com.

About Audentes Therapeutics, Inc.

Audentes™ is a biotechnology company committed to the development and commercialization of innovative new treatments for people with serious, rare muscle diseases through the application of adeno-associated virus (AAV) gene therapy technology. The company consists of a focused, experienced, and passionate team driven by the goal of improving the lives of patients. Audentes takes pride in strong, global relationships with the patient, research, and medical communities.

For more information regarding Audentes, please visit www.audentestx.com.

Contacts

REGENX Biosciences
Vit Vasista, 202-785-7438
vvasista@regenxbio.com
or
Audentes Therapeutics, Inc.
Matthew Patterson, 646-712-1001
mpatterson@audentestx.com