



Audentes Therapeutics Raises \$42.5 Million In Series B Financing

Deerfield, Sofinnova, and Venrock Join Existing Investors to Propel Leading Gene Therapy Company Through Significant Clinical and Corporate Development Milestones

SAN FRANCISCO, CA – December 2, 2014 – Audentes Therapeutics, Inc., a biotechnology company committed to the development and commercialization of gene therapy products for patients with serious, rare diseases, today announced the closing of a \$42.5 million Series B financing. Deerfield Management led the financing with participation from Sofinnova Ventures and Venrock. The company’s current investors, OrbiMed, 5AM Ventures, and Versant Ventures also fully participated. The additional funding will allow Audentes to achieve key clinical development milestones for its two lead programs and to continue research and in-licensing efforts to become a worldwide leader in gene therapy.

“The Audentes team is thrilled to work with such a prestigious group of investors who share our passion for bringing gene therapy products to patients as quickly as possible,” said Matthew Patterson, President and Chief Executive Officer of Audentes Therapeutics. “This financing will allow the company to rapidly advance our products for the treatment of X-Linked Myotubular Myopathy and Pompe Disease and to further expand our pipeline and internal capabilities as we pursue our mission to be a global leader in our field.”

Jonathan Leff, a Partner on the Private Transactions team at Deerfield, will become a member of the Audentes Board of Directors as a result of the financing.

About Audentes Therapeutics, Inc.

Audentes is a biotechnology company committed to the development and commercialization of gene therapy products for patients with serious, rare diseases. The company has two products in development, AT001 for the treatment of X-Linked Myotubular Myopathy (XLMTM) and AT002 for the treatment of Pompe Disease. Audentes completed a \$30 million Series A financing in July, 2013, led by OrbiMed with participation from 5AM Ventures and Versant Ventures. 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.

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 plays an important role in the development and maintenance of muscle cells. XLMTM affects approximately 1 in 50,000 newborn males worldwide. Audentes is developing AT001 for the treatment of XLMTM in collaboration with Genethon (www.genethon.fr). AT001 is a novel product based on AAV gene therapy technology.

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. AT002 is a novel product based on AAV gene therapy technology.

Contact

Audentes Therapeutics, Inc.

Jeffrey Gruis

jgruis@audentestx.com

415-638-6561