



Amsterdam Molecular Therapeutics Reports Promising Data from Cholesterol Lowering Gene Therapy Study

Gene Therapy Vector Successfully Delivers shRNA into Liver Cells

Amsterdam, The Netherlands, May 21, 2010 – Amsterdam Molecular Therapeutics (Euronext: AMT) a world leader in gene therapy, today [reported positive preclinical data from a study using an AAV gene therapy product to lower cholesterol](#). The data show that a single dose of the gene therapy carrying a short hairpin RNA to silence Apolipoprotein B100 (ApoB100), resulted in a reduction of serum cholesterol of approximately 80%, without any signs of toxicity. These data validate AMT's technology platform as a powerful tool for successful gene silencing within target cells. The data were presented by Annemart Koorneef, scientist at AMT, yesterday during the 13th Annual American Society of Gene and Cell Therapy (ASGCT) Meeting in Washington, DC.

"With just a single dose of our [cholesterol targeting gene therapy](#), a long-lasting, significant reduction of serum cholesterol is achieved. This preliminary study suggest that AMT's technology may have overcome one of the major problems of shRNA therapies, namely efficient and non-toxic intracellular delivery," noted Jörn Aldag, CEO of Amsterdam Molecular Therapeutics.

In the study, AMT used its proprietary AAV-based platform to efficiently deliver a shRNA that silences both human and mouse Apolipoprotein B100 (ApoB100). A single intravenous administration caused prolonged ApoB100 gene silencing that was sequence-specific and not associated with liver toxicity, oversaturation of the cellular miRNA machinery or induction of immune responses. In addition, it was shown that the shRNA constructs against ApoB100 specifically and efficiently silence human ApoB100 ex vivo.

ApoB100 is the structural protein of Low Density Lipoprotein (LDL) particles that carry cholesterol. Silencing of ApoB100 with shRNAs results in a reduction of LDL-cholesterol and has the potential to be used to treat hypercholesterolaemia and cardiovascular disease.

About Amsterdam Molecular Therapeutics

AMT, founded in 1998 and based in Amsterdam, is a leader in the development of human gene based therapies. Using adeno-associated viral (AAV) vectors as the delivery vehicle of choice for therapeutic genes, the company has been able to design and validate what is probably the first stable and scalable AAV production platform. This safe and efficacious proprietary platform offers a unique manufacturing capability which can be applied to a large number of rare (orphan) diseases that are caused by one faulty gene. Currently, AMT has a product pipeline with several AAV-based gene therapy products in LPL Deficiency, Hemophilia B, Duchenne Muscular Dystrophy, Acute Intermittent Porphyria and Parkinson's Disease at different stages of research or development.

AMT's lead product, Glybera, for the treatment of lipoprotein lipase deficiency has been filed for market authorization with the EMA. The current technology enables AMT to further expand its strategy to develop long-lasting therapies for severe metabolic diseases.

For further information

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subject to known and unknown risks and can be affected by factors that are beyond the control of AMT. Actual results could differ materially from current expectations due to a number of factors and uncertainties affecting AMT's business, including, but not limited to, the timely commencement and success of AMT's clinical trials and research endeavors, delays in receiving U.S. Food and Drug Administration or other regulatory approvals (i.e. EMEA, Health Canada), market acceptance of AMT's products, effectiveness of AMT's marketing and sales efforts, development of competing therapies and/or technologies, the terms of any future strategic alliances, the need for additional capital, the inability to obtain, or meet, conditions imposed for required governmental and regulatory approvals and consents. AMT expressly disclaims any intent or obligation to update these forward-looking statements except as required by law. For a more detailed description of the risk factors and uncertainties affecting AMT, refer to the prospectus of AMT's initial public offering on June 20, 2007, and AMT's public announcements made from time to time.