AMT Presents Positive Results of Phase I/II Study with Lead Product at Annual Meeting of American Society of Gene Therapy

Efficacy and Safety of Gene Therapy in Lipoprotein Lipase Deficiency Established

Amsterdam, The Netherlands – May 31, 2007 – Amsterdam Molecular Therapeutics (AMT), a leader in the field of human gene therapy, reported positive Phase I/II clinical trial results with its lead product AMT-010 (now referred to as AMT-011) in Complete Lipoprotein Lipase (LPL) deficient patients at the 10th Annual Meeting of the American Society of Gene Therapy (ASGT) in Seattle. The study's lead investigator is Erik S.G. Stroes, MD, PhD, from the University of Amsterdam Medical Center in The Netherlands.

Ronald Lorijn, CEO of AMT, said, "We believe these excellent results may represent an important milestone for the entire field of gene therapy and for the many patients who today cannot be treated. Our clinical success and the breakthrough in our production platform place us squarely at the forefront of the gene therapy field that is finally coming into its own."

AMT's products rely on a unique gene therapy platform that to date appears to circumvent many if not all of the obstacles that have prevented gene therapy to become a mainstay of clinical medicine. 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. As such, AMT's proprietary platform holds tremendous promise for thousands of rare (orphan) diseases that are caused by one faulty gene. AMT currently has a product pipeline with six products at different stages of development.

Study design
The primary goal of the study presented at the ASGT was to investigate if AMT-010 reduces the elevated triglyceride (fat) levels and the associated risk of pancreatitis in Complete LPL deficient patients. The study design was an eight-patient open label dose-escalating study (low and mid dose). The study was started in August 2005 and finalized in April 2007, and in effect demonstrates the safety and efficacy of AMT-010. All patients are now taking part in a follow-up trial, which will last for 12 months.

Efficacy
The primary endpoints of the study were either median fasting plasma triglyceride (TG) levels after administration of AMT-010 equal to or less than 10 mmol/L, or a 40% reduction in median fasting plasma TG after administration on top of a fat-free diet. For all subjects a substantial reduction of median TG levels was observed. Three patients (one from the low dose and 2 from the mid dose group) hit the primary endpoint, with TG levels below the target level of 10 mmol/L or a 40% reduction in TG levels. In two patients of the mid dose group the reduction of TG coincided with expression of active LPL in the injected muscle at 26-32 weeks after vector administration, which clearly demonstrates long-term expression of the therapeutic gene.

Safety

Furthermore, the study shows that AMT-010 was well-tolerated; there were no drug-related severe adverse events, and no dose-limiting toxicity. There was no measurable sign of local or systemic inflammation and no increase in creatinine phosphokinase concentrations, which implies that the impact on tissue function is limited and not harmful to humans.

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About AMT
Amsterdam Molecular Therapeutics BV (AMT) is a gene therapy company founded by scientists of the University of Amsterdam Medical Center (AMC) in 1998. AMT focuses on the development of gene-based therapies for orphan diseases. AMT's technology allows long-term gene expression and the specific delivery of therapeutic genes to target organs or tissues. AMT has optimized and validated the production of AAV-based gene therapy vectors. The result is a unique, stable, and scalable GMP production platform. Its lead product, AMT-011, is in pre-registration trials for the first indication: treatment of complete LPL deficiency. The company's Management, Supervisory Board and Scientific Advisory Board bring together an extensive know-how of genetics and the biotech and pharmaceutical industries.

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