AMT Accesses Technology for Treatment of Duchenne Muscular Dystrophy

Amsterdam, The Netherlands – May 22, 2008 – Amsterdam Molecular Therapeutics (Euronext: AMT), a leader in the field of human gene therapy, today announced that it obtained a license from La Sapienza University in Rome, Italy, to their advanced small nuclear RNA (snRNA)-based exon-skipping technology for the treatment of Duchenne muscular dystrophy (DMD). The combination with AMT's proprietary adeno-associated virus (AAV) gene therapy platform potentially makes up a long-term treatment for this seriously debilitating disease with a single administration of the product.

Duchenne Muscular Dystrophy

Duchenne muscular dystrophy, caused by mutations in the dystrophin gene (essential for muscle function) is a severely debilitating neuromuscular disease. It affects young children, is progressive and leads to death in young adulthood. In the USA and Europe about 120,000 people suffer from it. Today, there is no treatment to prevent the fatal outcome.

The group of Prof. Irene Bozzoni at La Sapienza has done seminal work to develop a treatment for DMD. Using adeno-associated viral vectors, AMT's gene therapy platform of choice, and an exon-skipping snRNA program Bozzoni and co-workers demonstrated long term systemic and therapeutic effect in animals after a single administration[1]. One of the important advantages of the Bozzoni technology is that a single systemic delivery of the AAV vector containing the required antisense RNA-coding construct will result in long-term treatment of Duchenne muscular dystrophy.

Formalizing relationship with leading RNA research group of prof. Irene Bozzoni

Ronald Lorijn, CEO of AMT said: "We are very excited to have inlicenced an exciting therapeutic approach, which has shown to provide a life-long cure in rodents suffering from Duchenne's disease. In Duchenne's disease in particular the combined application of snRNA and AAV vectors has shown tremendous promise. Access to La Sapienza's RNA technology perfectly complements our gene and vector therapy platform. It adds a project to AMT's R&D pipeline that will start its pre-clinical phase this year."

Exon skipping in DMD

Exon skipping is a technology to neutralize genetic defects by preventing the faulty parts of the gene being used. At the cellular level, a molecule called messenger RNA (mRNA) reads off (transcribes) the protein instructions from the gene. It then transports these instructions to the ribosomes in the cell where the protein is assembled. Messenger RNA is not a transcript of the complete gene sequence, but only of the exons, which are the sections of the gene that code for a portion of the protein. If one of the exons contains an error, this process may be halted, and the production of the full-length dystrophin protein cannot take place. However, when the faulty exon is eliminated (i.e. skipped), protein synthesis does take place and leads to a functional, albeit shorter, dystrophin protein. By using the exon-skipping technique the mutated exons in the dystrophin mRNA that contain errors are "skipped" and as a result the muscle cells are able to produce functional dystrophin protein.

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[1] Denti, M.A., Rosa, A., D'Antona, G., D'Antona, G., Sthandier, O., De Angelis, F.G., Nicoletti, C., Allocca, M., Pansarasa, O., Parente, V., Musarò, A., Auricchio, A., Bottinelli, R., and Bozzoni, I. (2006), Body-wide gene therapy of Duchenne muscular dystrophy in the *mdx* mouse model, *Proceedings of the National Academy of Sciences of the United States of America*, 103: 3758-3763.

De Angelis, F.G., Sthandier, O., Berarducci, B., Toso, S., Galluzzi, G., Ricci, E., Cossu, G., and Bozzoni, I. (2002) Chimeric snRNA molecules carrying antisense sequences against the splice junctions of exon 51 of the dystrophin pre-mRNA induce exon skipping and restoration of a dystrophin synthesis in Δ 48-50 DMD cells, *Proceedings of the National Academy of Sciences of the United States of America*, 99: 9456-9461.

Denti, M.A., Rosa, A., D'Antona, G., D'Antona, G., Sthandier, O., De Angelis, F.G., Nicoletti, C., Allocca, M., Pansarasa, O., Parente, V., Musarò, A., Auricchio, A., Bottinelli, R., and Bozzoni, I. (2006), Chimeric Adeno-Associated Virus/Antisense U1 Small Nuclear RNA Effectively Rescues Dystrophin Synthesis and Muscle Function by Local Treatment of *mdx* Mice, *Human Gene Therapy*¸ 17: 565–574.

About Amsterdam Molecular Therapeutics

AMT has a unique gene therapy platform that to date appears to circumvent many if not all of the obstacles that have prevented gene therapy from becoming 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.

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Certain statements in this press release are "forward-looking statements" including those that refer to management's plans and expectations for future operations, prospects and financial condition. Words such as "strategy," "expects," "plans," "anticipates," "believes," "will," "continues," "estimates," "intends," "projects," "goals," "targets" and other words of similar meaning are intended to identify such forward-looking statements. Such statements are based on the current expectations of the management of Amsterdam Molecular Therapeutics only. Undue reliance should not be placed on these statements because, by their nature, they are 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.