



## **AMT Successful in Preclinical Treatment of DMD**

**Amsterdam, The Netherlands – November 11, 2009** – Amsterdam Molecular Therapeutics (Euronext: AMT), a leader in the field of human gene therapy, announced today that it has successfully treated Duchenne muscular dystrophy (DMD) in an animal model with its proprietary gene therapy. The proof of concept studies were performed in collaboration with the group of Professor Irene Bozzoni (University of Rome, La Sapienza, Italy) and demonstrated effectiveness in the heart as well as in skeletal muscles. In a previous study, AMT's gene therapy approach was shown to be successful in the treatment of diseased human muscle cells obtained from biopsies of DMD patients. These data establish a robust basis for AMT's therapeutic approach to DMD.

"We are proud to establish proof of concept with our gene therapy for Duchenne muscular dystrophy, as it is an important new step in developing a treatment for this progressive and devastating disease," said Jörn Aldag, Chief Executive Officer of AMT. "In particular, our positive results in the heart and respiratory muscles bring hope that we might be able to prevent the fatal outcome of this disease in the future, potentially through a long-term remedy with a single treatment."

### **About Duchenne Muscular Dystrophy**

Duchenne muscular dystrophy is a severe disease characterized by progressive muscle degeneration. It affects young children, almost exclusively boys, and leads to paralysis and death in young adulthood. The disease is caused by mutations in the dystrophin gene, as a result of which the production of functional dystrophin protein, an important structural component within muscle tissue, is blocked. Currently, there is no treatment to prevent the fatal outcome of this disease. DMD affects one in 3,500 males, making it the most prevalent of muscular dystrophies.

AMT is developing a gene therapy product for DMD based on 'exon skipping' technology which results in bypassing the genetic defect such that the functional protein can be formed again. Positive long-term therapeutic effects of this approach have been demonstrated in animals.

### **About Amsterdam Molecular Therapeutics**

AMT has a unique gene therapy platform that 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, especially diseases that are caused by one faulty gene. Currently, AMT has a pipeline with nine products at different stages of research or development.

### **For information**

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*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.*