AMT receives orphan drug designation from the U.S. Food and Drug Administration Duchenne Muscular Dystrophy gene therapy

Amsterdam, The Netherlands – September 22, 2010 – Amsterdam Molecular Therapeutics (AMT) Holding N.V. (Euronext: AMT), a leader in the field of human gene therapy, announced today that the U.S. Food and Drug Administration (FDA) has designated AMT-080, a gene therapy for Duchenne muscular dystrophy (DMD) as an orphan drug. In October 2009, the Committee for Orphan Medical Products of the European Medicines Agency granted AMT-080 orphan designation for the same indication in the European Union.

AMT has shown efficacy in studies of a preclinical model of DMD. These proof of concept studies demonstrated that AMT's technology resulted in functional dystrophin synthesis in both the heart and skeletal muscles, leading to the prevention of muscular dystrophy. These data are strengthened by a study in which this gene therapy approach was shown to successfully restore dystrophin activity in diseased human muscle cells obtained from biopsies of DMD patients. A Phase I/II clinical trial is scheduled to start by the end of 2012.

AMT has received an Innovation Credit of up to € 4 million from the Dutch government to support the development of AMT's gene therapy treatment for Duchenne Muscular Dystrophy (DMD). The credit is granted by SenterNovem, an agency of the Dutch Ministry of Economic affairs.

"It is exceptional that we have been able to reveal the promise of this therapy to the FDA in this early stage of the development. We believe our proven adenoassociated viral vector technology used in all our gene therapy products provides a distinct advantage. AMT has successfully conducted three clinical trials with its lead product Glybera that employs this technology, confirming that AAV-based delivery technology is safe and efficacious," noted Jörn Aldag, CEO of Amsterdam Molecular Therapeutics.

The FDA's orphan drug designation is intended to encourage research and development of new therapies for diseases that affect fewer than 200,000 U.S. residents. As a designated orphan drug, AMT-080 is eligible for tax credits based on its clinical development costs, as well as assistance from the FDA in guiding the drug through the regulatory approval process. The designation provides the opportunity for AMT-080 to obtain market exclusivity for seven years from the drug's marketing approval date.

About Duchenne Muscular Dystrophy

DMD is a severe disease characterized by progressive muscle degeneration. It affects young children, almost exclusively boys, and leads to progressive 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 one of the most prevalent of muscular dystrophies.

About Amsterdam Molecular Therapeutics (AMT) Holding N.V.

AMT, founded in 1998 and based in Amsterdam, is a leader in the development of human gene based therapies. Using AAV 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 LPLD, Hemophilia B, DMD, Acute Intermittent Porphyria and Parkinson's Disease at different stages of research or development.

For further enquiries:

Ellen Roest
AMT Communications
Mobile +31 6 2900 6179
e.roest@amtbiopharma.com

Mike Sinclair
Halsin Partners
Tel +44 20 7318 2955
msinclair@halsin.com

Jörn Aldag CEO
Tel +31 20 566 7394
j.aldag@amtbiopharma.com

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 (AMT) Holding N.V. 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. EMA, 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.