AMT Receives Grant from Dutch Parents Organization for Duchenne Muscular Dystrophy Gene Therapy

Amsterdam, The Netherlands - March 7, 2011 -
Amsterdam Molecular Therapeutics (AMT) (Euronext: AMT), a leader in the field of human gene therapy, announced today that the Duchenne Parent Project, based in the Netherlands, has awarded AMT a grant of EUR 145,000 to support the development of AMT-080, AMT's gene therapy for Duchenne Muscular Dystrophy (DMD). DMD is a severe, fatal disease affecting young children, almost exclusively boys, characterized by progressive muscle degeneration. It is caused by mutations in the dystrophin gene, which blocks the production of functional dystrophin protein, an important structural component within muscle tissue. The Duchenne Parent Project was established by the parents of children with DMD to provide information and vital support for families affected by the disease.

AMT-080 is based on 'exon skipping' technology, which effectively bypasses the genetic defect so that functional dystrophin protein can be produced. AMT plans to begin a Phase I/II trial with AMT-080 by the end of 2012 following successful preclinical studies where AMT-080 has shown efficacy in models of DMD. These proof of concept studies demonstrated that AMT-080 produced functional dystrophin synthesis in both the heart and skeletal muscles, leading to the prevention of muscular dystrophy in these models. 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.

"Support such as this grant received from the Duchenne Parent Project, as well as other similar patient-led organizations, is greatly appreciated by the whole team at AMT. We are all committed to progressing this treatment into clinical trials as soon as possible and ultimately making it available to patients," said Jorn Aldag, Chief Executive Officer of AMT. "This generous grant and the support previously given by SenterNovem has ensured priority development for this innovative potential treatment for DMD within AMT."

In January 2010 Agentschap NL(formerly SenterNovem), an agency of the Dutch Ministry of Economic Affairs, awarded AMT an Innovation Credit of up to EUR 4 million, and in October 2009 and September 2010 AMT received Orphan Drug designation from the European Medicines Agency and the US Food and Drug Administration respectively.

About Duchenne Muscular Dystrophy (DMD)
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 is a world leader in the development of human gene based therapies. The company's lead product Glybera(R), a gene therapy for the treatment of lipoprotein lipase deficiency (LPLD), is currently under review by the European Medicines Agency (EMA). If approved, Glybera will be the first gene therapy product to be marketed in Europe. AMT also has a product pipeline of several gene therapy products in development for hemophilia B, Duchenne Muscular Dystrophy, acute intermittent porphyria, and Parkinson's disease. Using adeno-associated viral (AAV) derived vectors as the delivery vehicle of choice for therapeutic genes, the company has been able to design and validate probably the world's first stable and scalable AAV manufacturing platform. This proprietary platform can be applied to a large number of rare (orphan) diseases caused by one faulty gene and allows AMT to pursue its strategy of focusing on this sector of the industry. AMT was founded in 1998 and is based in Amsterdam. Further information can be found at www.amtbiopharma.com.

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