AMT Receives € 1.1 Million Funding for Acute Intermittent Porphyria Gene Therapy as part of EU Consortium Grant

Amsterdam, The Netherlands – January 31, 2011 – Amsterdam Molecular Therapeutics (Euronext: AMT), a leader in the field of human gene therapy, announced today that the European Union (EU) has finalized a € 3.3 million grant to the AIPGENE consortium, of which AMT is a member, for the development of a gene therapy product for Acute Intermittent Porphyria (AIP). AIP is a severe and progressive disease caused by the inability of the body to produce the heme protein, a component of hemoglobin, as well as other important proteins.

From the grant, made under the EU's FP7 program, AMT will receive € 1.1 million. The grant will cover approximately 75% of AMT's overall development costs to bring this product forward to completion of a Phase I/II study in humans. AMT holds the commercialization rights to the AIP gene therapy (AMT-021). The AIPGENE consortium is led by Fundación para la Investigatión Médica Aplicada (FIMA, Spain), and in addition to AMT, it includes Clínica Universidad de Navarra (Spain), Stockholms Läns Landsting (Sweden), Deutsches Krebsforschungszentrum (Germany), Digna Biotech (Spain) and Servicio Madrileño de Salud (Spain).

"This grant allows us to prioritize the development of our AIP gene therapy program for this progressive and devastating disease within AMT's pipeline, as well as initiating the next step in advancing a potentially more effective, long-term treatment for patients," said Jörn Aldag, Chief Executive Officer of AMT.

AIP results from mutations in the PBDG gene, which encodes for the enzyme porphobilinogen deaminase, a liver protein necessary for the production of heme. AMT-021 is intended to provide long-term normalization of the PBGD protein in order to prevent acute attacks and their complications. AMT has demonstrated that AMT-021 produces this normalization of the PBGD protein in an animal model of AIP. In addition, it completely prevented the occurrence of attacks and significantly ameliorated the neuropathy that develops in untreated animals. AMT's partner at FIMA has shown persistence of expression of genes in the liver for more than a year using AAV-mediated delivery methods similar to AMT-021. With the support of the all the AIPGENE partners, AMT anticipates AIP patient enrolment in a clinical trial to begin in 2012.

On 28 May 2008, the European Medicines Agency (EMA) granted Orphan Drug Designation to AMT's gene therapy product for the treatment of AIP. This entitles AMT to ten years of market exclusivity to treat AIP in Europe following marketing approval, provided that this product candidate is the first such approved new drug with a major medical benefit.

About Acute Intermittent Porphyria
Acute intermittent porphyria is a rare genetic disease where mutations in the porphobilinogen deaminase (PBGD) gene, results in insufficient activity of a protein necessary for heme synthesis. This leads to the accumulation of toxic intermediate metabolites that produce a wide variety of problems including acute and severe abdominal pains, psychiatric and neurological illnesses. There is currently no cure for this condition and the disease is typically progressive.

About Amsterdam Molecular Therapeutics
AMT is a world leader in the development of human gene based therapies. The company's lead product Glybera®, 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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