



Start of Phase I/II Gene Therapy Clinical Trial for Hemophilia B

Amsterdam, The Netherlands – March 10, 2010 – Amsterdam Molecular Therapeutics (Euronext: AMT), a leader in the field of human gene therapy, announced today that the first patient has been dosed in the Phase I/II exploratory clinical trial with a gene therapy product for hemophilia B, a seriously debilitating and potentially lethal disease.

The trial is an open label dose-escalation study using a vector-gene combination developed at the renowned St. Jude Children's Research Hospital. Dr. Arthur W. Nienhuis of St. Jude is the principal investigator of the ongoing trial. The work was initiated at St. Jude more than a decade ago by Drs. Andrew Davidoff and Amit Nathwani and the collaboration has continued following Dr. Nathwani's return to London. The collaboration involves St. Jude and University College London and other institutions in the US and Britain. The objective of the trial is to assess the safety and efficacy of different doses of hemophilia B gene therapy. Hemophilia B is an inherited condition in which patients may have repeated and sometimes life threatening bleeds after accidental trauma or medical interventions, because they do not have sufficient functioning of an essential blood clotting factor, called Factor IX.

AMT will build on the outcome of this exploratory trial and is preparing for additional clinical development to establish safety, tolerability and proof-of-concept with Factor IX gene therapy produced using AMT's proprietary, clinically validated production system. AMT has the exclusive commercialization rights to the Factor IX gene used in the St. Jude trial and has the ability to produce gene therapy product for hemophilia B at high quality on a commercial scale. Additional developmental work using AMT's production system is on-going at St. Jude with AMT support.

Jörn Aldag CEO of AMT said: "Dr. Andrew Davidoff and his group at St. Jude, together with Professor Nathwani in London, have done very important scientific work on hemophilia B. We are really looking forward to the results of the trial for continuing our collaboration, aiming for a real cure for patients with this bleeding disorder. Use of the Factor IX gene fits perfectly with AMT's proprietary gene therapy platform and our business strategy of developing cures for seriously debilitating orphan diseases. "

This hemophilia B gene therapy, administered once, will introduce the functional gene for the Factor IX protein into the patient's liver cells with the goal to restore blood clotting functionality long-term. In pre-clinical studies, Factor IX gene therapy resulted in long-term production of Factor IX protein at a therapeutically significant level after a single administration. If this approach is successful, the long term efficacy of one time administered hemophilia B gene therapy is expected to be perceived as a significant advance over the current regular dosing of recombinant Factor IX. In addition, the efficacy profile of this gene therapy is anticipated to exceed that of current therapy, as the gene therapy should lead to stable Factor IX levels whereas current recombinant protein treatment causes peaks and troughs. It is hoped that hemophilia B gene therapy, therefore, can potentially replace all recombinant Factor IX products.

The UK Medicines and Healthcare products Regulatory Agency as well as the US Food and Drug Administration have approved the current trial.

About the Disease

Hemophilia B is characterized by severe episodes of external and internal bleeding, resulting in significant morbidity. The episodes cause long-term damage, for instance to the joints, and may be fatal if they occur in the brain. The defect in blood clotting in hemophilia B is caused by the absence of functional clotting Factor IX as a result of mutations in the gene encoding this protein. The factor IX gene is located on the X chromosome. It is an X-linked recessive trait, which explains why only males are usually affected.

Hemophilia B is a rare disease, occurring in 1 in 30,000 people, almost always in males. The total number of patients in Europe and the USA together is estimated to be between 35,000 and 40,000.

Currently, frequent intravenous administrations of recombinant Factor IX are required to stop or prevent bleeding. Protein replacement therapy is costly, cumbersome, and does not completely prevent bleeding.

About Amsterdam Molecular Therapeutics

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 information

AMT will be presenting at Bio Europe Spring 2010 Conference at the Centre Convencions Internacional, Barcelona, at 1200 (CET) on Wednesday March 10, 2010.

Jörn Aldag

CEO

Tel +31 (0)20 566 7394

Tel +31(0)6 8195 3060

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 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.