EMA starts formal review of Glybera® dossier

Amsterdam, The Netherlands – January 25, 2010 – Amsterdam Molecular Therapeutics (Euronext: AMT), a leader in the field of human gene therapy, has reached another important milestone in the official marketing authorisation process for its lead product Glybera®, AMT's proprietary product for lipoprotein lipase deficiency (LPLD). The submission of the Glybera® Marketing Authorisation Application (MAA), announced earlier, has cleared the validation stage with The European Medicines Agency (EMA, formerly known as EMEA). The EMA will now commence its formal review of Glybera®.

AMT has concluded two clinical studies for LPLD, in Europe and Canada, and long term follow-up from both of these is ongoing, as is a third clinical study in Canada. In these three studies Glybera® has shown a sizeable decrease in the incidence of pancreatitis, or acute inflammation of the pancreas, the most debilitating complication of LPLD. In addition, these studies indicate that Glybera® has an excellent safety profile.

"The acceptance of the Glybera® dossier by EMA is a significant step towards marketing approval for Glybera®. Moreover, it demonstrates AMT's development capability. A future approval of the MAA for Glybera would fully validate our gene therapy approach and our adeno-associated viral (AAV) vector delivery platform. We believe this step offers hope to many patients, as gene therapy may become the therapeutic approach of choice for inherited disorders" said Jörn Aldag, Chief Executive Officer of AMT.

The EMA formal review will be conducted via the centralised procedure, which is the standard route for all advanced therapies. During 2010, AMT expects to provide further updates on the results from its follow-up and ongoing studies, in accordance with reporting regulations.

About the Disease
LPLD is a seriously debilitating, and potentially lethal, orphan disease for which no treatment exists today. The disease is caused by mutations in the LPL gene, resulting in highly decreased or absent activity of LPL protein in patients. This protein is needed in order to break down large fat-carrying particles that circulate in the blood after each meal. When such particles, called chylomicrons, accumulate in the blood, they may obstruct small blood vessels. This can result not only in pancreatitis, but also in difficult-to-treat diabetes, and is associated with significant morbidity and mortality.

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 LPL Deficiency, Hemophilia B, DMD, Acute Intermittent Porphyria and Parkinson's Disease at different stages of research or development.

For information

AMT will be presenting at the BioCEO & Investor Conference, Waldorf-Astoria, New York City, at 9:30 am (EST) on Tuesday, February 9, 2010.

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