



## **AMT intends to incorporate additional data to its Marketing Authorization Dossier for Glybera®**

**Amsterdam, The Netherlands – 3 October, 2008** – Amsterdam Molecular Therapeutics (Euronext: AMT), a leader in the field of human gene therapy, announced today that it intends to incorporate additional data into the Marketing Authorization Application (MAA) for Glybera® for Lipoprotein Lipase Deficiency (LPLD), a seriously debilitating and potentially lethal disease.

The data obtained so far from two phase II clinical studies (22 patients in total) demonstrate that Glybera® is well-tolerated (no drug-related severe adverse events and no dose-limiting toxicity). Positive effects were shown on fat (triglyceride) metabolism, resulting in an almost complete disappearance of pancreatitis during both the immediate 12-week study observation period and the long-term follow-up (up to 3 years post-injection), as well as the disappearance or reduction of fat accumulations in skin or retina. All patients reported gain of energy and in the two patients that had diabetes, a reduction of insulin resistance was observed, leading to a reduction of their diabetic medication.

In consultation with the Rapporteur countries, Germany and the United Kingdom, of the European Medicines Agency (EMA) and Health Canada, AMT has decided to expand the clinical experience and to augment the Marketing Authorization Application (MAA) with an additional 16-patient study that will investigate the broader impact of this gene therapy on additional aspects of lipid metabolism. Preparations for this study to be conducted in Canada will start immediately. Submission of the MAA to the EMA is foreseen in the second half of 2009. In line with the guidance given in the half year financial report, the Company's cash position allows the completion of this study to be done with existing financial resources.

AMT has developed Glybera® as a cure for patients with the rare genetic disorder, LPLD. Because of a defective gene, LPLD patients do not produce an enzyme that normally breaks down fats in the blood. These patients have extremely high fat levels in their blood, resulting in recurrent and potentially lethal pancreatitis as well as an increased risk of cardiovascular complications and diabetes. Currently, there exists no effective treatment or cure for this serious disease.

Glybera® is a gene therapy, which inserts into muscle tissue a healthy gene that restores the enzyme (lipoprotein lipase) activity required to process fat. The advantage of this therapy is its potential to cure a disease instead of just treating the symptoms.

### **About Amsterdam Molecular Therapeutics**

AMT has a unique gene therapy platform that to date appears to circumvent many if not all of the obstacles that have prevented gene therapy from becoming a mainstay of clinical medicine. Using adeno-associated viral (AAV) vectors 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. As such, AMT's proprietary platform holds tremendous promise for thousands of rare (orphan) diseases that are caused by one faulty gene. AMT currently has a product pipeline with seven products at different stages of development.

### **For information**

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*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.*