

Amsterdam Molecular Therapeutics B.V

07.05.2009 - 07:07 Uhr

AMT Starts Preregistration Trial for Glybera(TM)

Amsterdam (ots/PRNewswire) -

Amsterdam Molecular Therapeutics (Euronext: AMT), a leader in the field of human gene therapy, announced today the treatment of the first patient in a preregistration clinical trial with Glybera(TM). This gene therapy product targets lipoprotein lipase deficiency (LPLD), a seriously debilitating and potentially lethal disease.

The randomized controlled trial has been designed to gather additional data on the effects of Glybera on lipid metabolism and the mechanisms underlying the prevention of pancreatitis attacks. The trial is being performed under a Clinical Trial Application approved by Health Canada.

The new clinical trial builds on positive data obtained from two previous clinical trials in which a total of 22 LPLD patients were treated. These data indicate that a single treatment with Glybera results in a long-term, statistically significant and clinically important reduction in the incidence of acute pancreatitis in LPLD patients. The longest follow-up of individual patients is well over three years, and the cumulative follow-up of all patients is more than 45 years. The therapy was well tolerated and no drug-related severe adverse events or unexpected side-effects have been observed.

AMT will include the data from the new trial in the Marketing Authorization Application for Glybera. The submission of the dossier to the European Medicines Agency is planned for the second half of 2009.

About the Disease

LPLD is an 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 results in recurrent and severe acute inflammation of the pancreas, called pancreatitis, the most debilitating complication of LPLD. The disease can result in difficult-to-treat diabetes and is associated with significant morbidity and mortality.

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, especially those that are caused by one faulty gene. Currently, AMT has a product pipeline with nine products at different stages of

development.

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.

Contact:

For Information, André Verwei, CFO, Tel +31(0)20-5667394,
a.verwei@amtbiopharma.com

Diese Meldung kann unter <https://www.presseportal.ch/de/pm/100015218/100582673> abgerufen werden.