

Amsterdam Molecular Therapeutics B.V

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AMT's Cooperative Research and Development Agreement With NIH to Boost Production Capacity

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- Production Increase Brings Large Disease Areas Within Reach

Amsterdam Molecular Therapeutics (Euronext: AMT), a leader in the field of human gene therapy, today announced that it obtained a Cooperative Research and Development Agreement from the National Heart, Lung, and Blood Institute (NHLBI) of the National Institutes of Health (NIH), Bethesda, Maryland, that promises to substantially enhance the power of AMT's gene therapy platform.

The Agreement

The Cooperative Research and Development Agreement (CRADA) with the NIH gives AMT the option to license the exclusive rights to the recombinant adeno-associated virus (rAAV) baculoviral manufacturing technology developed for treating Duchenne Muscular Dystrophy (DMD) by Robert Kotin, Ph.D., and colleagues at the NHLBI Laboratory of Biochemical Genetics. While AMT's current production platform is based on a 50-liter bioreactor set-up, the application of the NIH technology has been scaled-up to 250 liters, and allows for further expansion to 1,000 liters. These production levels are unique in the gene therapy field today and are relevant in the context of some of the products that AMT has under development that affect large patient populations, such as Factor IX for hemophilia B, IGF-I for liver cirrhosis, and AMT-011 for NASH. The scale-up is also important for treatment of Duchenne Muscular Dystrophy (DMD) for which AMT has just closed an agreement with La Sapienza University in Rome, Italy, since this indication requires a relatively high dose of gene therapy vector in order to correct the defect in all affected muscles.

Ronald Lorijn, CEO of AMT said: "This CRADA with the NIH provides AMT with a technology that in combination with our proprietary in-house platform adds a whole new dimension to our manufacturing platform. Our combined effort has the potential to greatly increase the therapeutic reach of our gene therapies to benefit far larger patient groups than was hitherto thought possible. In addition, it would allow us to attempt the treatment of diseases that require the systemic (as opposed to local) expression of therapeutic genes. Duchenne Muscular Dystrophy is such a disease, and together with La Sapienza in Rome we will work very hard to develop a treatment for this wasting disorder for which there is currently no treatment."

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 six 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.

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