

## Amsterdam Molecular Therapeutics BV (AMT) Acquires License to AAV Patents from Targeted Genetics Corporation

License forms a critical component of AMT's commercialisation strategy for lead candidate AMT-011

AMSTERDAM, The Netherlands – December 12, 2006: Amsterdam Molecular Therapeutics BV (AMT), a world leader in gene therapy, today announced it has acquired a non-exclusive license to two patents covering AAV1 (adeno-associated virus) vectors from Targeted Genetics Corp. (NASDAQ:TGEN). The license allows AMT to develop and commercialise the therapeutic gene product AMT-011 for treating lipoprotein lipase (LPL) deficiencies type I and type V.

In preclinical studies, AMT has demonstrated effective replacement of LPL gene function, leading to complete normalisation of triglyceride levels in two different LPL deficient models. In 2004 the EMEA assigned "orphan drug" status to this LPL gene product. AMT-011 has entered phase I/II clinical stage.

"This license is a key step in AMT's commercialisation strategy of its lead drug candidate AMT-011. This breakthrough gene therapy will allow AMT to provide patients suffering from a serious, debilitating and often life-threatening metabolic disease, caused by a monogenetic defect, a fundamental cure", said Ronald H.W. Lorijn, MD, PhD, M.B.A., Chief Executive Officer of AMT.

H. Stewart Parker, president and chief executive officer of Targeted Genetics said, "This agreement allows us to participate, financially, in the commercialisation of AAV therapeutics by others. We are truly seeing the expansion of interest in AAV-based molecular medicines as a therapeutic platform. AMT-011 could be the first commercially available AAV-based therapeutic and may provide us with a relatively near-term milestone and royalty revenue stream to support our internal product development efforts. We wish AMT success as they move this therapeutic candidate through development."

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## **About LPL Deficiency**

LPL deficiency is an autosomal recessive inherited condition caused by homozygosity or compound heterozygosity for mutations in the LPL gene. LPL is the principal enzyme involved in the clearance of triglycerides from the bloodstream. Affected patients develop chronic pancreatitis, ultimately resulting in diabetes mellitus. The dysregulation in lipid metabolism that occurs in LPL deficiency also predisposes affected patients to cardiovascular diseases, including

coronary artery disease and heart attack. Due to the lack of LPL activity, these patients have extremely high triglyceride (TG) levels in their plasma and suffer from (potentially lethal) pancreatitis. As there is currently no drug or any specific therapy available to modulate the course of the illness, these patients are at high risk of morbidity and mortality. Currently, the only effective treatment for the condition is a severe reduction in the consumption of dietary fat, a regimen to which many patients have difficulty adhering. AMT estimates the worldwide population of LPL type 1 deficiency at 4,000 patients and LPL type V deficiency at approximately 100,000.

## **About AMT**

Amsterdam Molecular Therapeutics BV is a fully integrated gene therapy company founded by scientists of the University of Amsterdam Medical Center (AMC) in 1998. AMT focuses on the development of gene-based therapies for orphan metabolic, central nervous system and ocular diseases. AMT's long-term gene expression technology is based on specific delivery of therapeutic genes into target organs or tissues. Production of AAV-based gene therapy vectors has been optimized and AMT has developed and validated a unique, stable and scalable GMP production platform. Its lead product, AMT-011, is in phase II for the first indication: treatment of lipoprotein lipase deficiency type I. For further information, visit the Company's website at www.amtbv.com

## **About Targeted Genetics**

Targeted Genetics Corporation is a biotechnology company committed to the development of innovative targeted molecular therapies for the prevention and treatment of acquired and inherited diseases with significant unmet medical need. Targeted Genetics' proprietary Adeno-Associated Virus (AAV) technology platform allows it to deliver genes coding proteins to increase gene function, as well as RNAi to decrease or silence gene function. Targeted Genetics' product development efforts target inflammatory arthritis, AIDS prophylaxis, congestive heart failure and Huntington's disease. To learn more about Targeted Genetics, visit the Company's website at www.targetedgenetics.com.