



## **Amsterdam Molecular Therapeutics BV receives global rights to develop and commercialize AMT-020 for acute intermittent porphyria**

*Rights from University of Navarra, Pamplona, Spain  
strengthen AMT's portfolio of gene therapy products*

**Amsterdam, the Netherlands, February 28 2007** – Amsterdam Molecular Therapeutics BV (AMT), a leader in the field of human gene therapy, announces that it has obtained exclusive worldwide rights to develop and commercialise AMT-020 as a therapeutic product from UTE CIMA, Proyecto de Biomedicina CIMA, S.L. and Digna Biotech (all parties part of the University of Navarra, Pamplona, Spain).

The licence allows AMT to initiate the clinical development of AMT-020 to treat acute intermittent porphyria.

This follows successful collaboration between CIMA and AMT, demonstrating the safety and preclinical efficacy of the product, AMT-020. AMT-020 is an AAV vector gene therapy containing the porphobilinogen deaminase gene which encodes for the enzyme that is defective in acute intermittent porphyria. This disease is associated with recurrent attacks of abdominal pain, gastrointestinal dysfunction, and neurologic disturbances.

AMT-020 builds the Company's gene therapy portfolio. AMT's lead product is AMT-011, in phase II trials for the treatment of genetic lipoprotein lipase (LPL) deficiency type I.

### **About AMT**

Amsterdam Molecular Therapeutics BV (AMT) is a 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 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. The company's Management, Supervisory and Scientific Advisory Board bring together an extensive know-how from both gene science as well as the biotech and pharmaceutical worlds. For further information, go to [www.amtbv.com](http://www.amtbv.com).

### **About Acute Intermittent Porphyria**

Acute intermittent porphyria is an inherited disease characterised by attacks of acute abdominal pain, muscular weakness and a complex array of neurovisceral and psychiatric symptoms that may be life threatening if treated incorrectly. There is also an increased risk of primary liver cancer and renal failure. The attacks are normally triggered when the heme synthesis is induced by infections, stress, alcohol, hormones, or specific drugs. It is estimated that prevalence of the

disease can be as high as 60-100 cases per 100,000 population in northern Sweden. Women are more commonly affected than men

Acute intermittent porphyria is a metabolic error in heme biosynthesis in which the porphobilinogen deaminase (PBGD) is partially deficient by inactivating mutations in one allele. The condition is inherited in an autosomal dominant pattern with incomplete penetrance. The prevalence of symptomatic disease is 1–2 per 20,000 individuals, and women are more frequently affected than men.

**For further information, please contact:**

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