uniQure

Corporate Overview

Founded: 1998 in the Netherlands as Amsterdam Molecular Therapeutics (AMT)
Listed on NASDAQ: February 5, 2014 (QURE)
Full-time employees: Approximately 200 in Amsterdam, the Netherlands and Lexington, Massachusetts
Chief Executive Officer: Matthew Kapusta

Our focus is to advance the future of medicine through gene therapy.

uniQure is delivering on the promise of gene therapy, single treatments with potentially curative results. We have developed a modular technology platform to rapidly bring new disease-modifying therapies to patients with severe genetic diseases.

We have a focused pipeline of innovative gene therapies and have established clinical proof-of-concept in our lead indication, hemophilia B, and preclinical proof-of-concept in Huntington's disease. Our pipeline of adeno-associated virus (AAV)-based gene therapies has been developed using an innovative technology platform, supported by industry-leading proprietary commercial-grade manufacturing capabilities.

uniQure has built an industry-leading AAV gene therapy platform.

Defective genes and improper protein function are the cause of many diseases. Gene transfer, using adeno-associated viruses, or AAVs, is designed to provide copies of a therapeutic gene in a "gene cassette" to correct the defective or missing protein, restoring function or silencing a disease-causing effect in target cells. The goal: a single administration with potentially long-term or curative benefits.

At uniQure, we utilize an AAV vector called AAV5 to deliver the gene cassette to the target tissue. We have presented preclinical data indicating that, in contrast with other AAV vectors, pre-existing anti-AAV neutralizing antibodies do not interfere with successful gene transfer when AAV5 is used. This significantly expands the number of patients who potentially could benefit from our gene therapies.

Paired with our state-of-the-art manufacturing process, uniQure brings together all key elements for a successful gene therapy product.

State-of-the-Art Manufacturing Capability

uniQure produces our AAV-based gene therapies in our own facilities using insect cells and baculoviruses, a common family of harmless viruses found in invertebrates. uniQure has worked in insect cell baculovirus manufacturing for well over a decade and has established a very strong and unique patent position in fundamental aspects of the technology that are applicable to the entire industry.

Our facility in Lexington, Massachusetts is one of the largest, most versatile gene therapy manufacturing facilities in the world. uniQure has made significant investments in designing, constructing and equipping our 55,000 square-foot facility with state-of-the-art laboratories and commercial-scale production capabilities. The facility offers GMP production capabilities to support all of our existing programs, with flexibility to expand further.

A focused pipeline of innovative gene therapies

Hemophilia B
uniQure is developing a gene therapy for hemophilia B, a severe orphan blood clotting disorder. Our gene therapy product candidate AMT-061 consists of an AAV5 vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX-Padua).

Our goal with AMT-061 is to give all people living with hemophilia B access to a one-time treatment capable of normalizing FIX activity and eliminating the need for replacement therapy, without the risk of immune responses. In February, uniQure announced updated clinical data in patients treated with AMT-061 showing that therapeutic levels of Factor IX (FIX) activity have been sustained in all patients up to sixteen weeks after a single administration -- with increases in FIX activity of up to 51% and a mean of 38% -- exceeding the levels generally considered sufficient to eliminate or significantly reduce the risk of bleeding events. uniQure is currently enrolling patients in our Phase III HOPE-B pivotal study. We have treated the first patient in the trial and plan to complete enrollment this year.

Huntington's Disease
uniQure is developing a gene therapy for Huntington's disease, a rare, inherited neurodegenerative disorder that leads to loss of muscle coordination, behavioral abnormalities and cognitive decline, resulting in complete physical and mental deterioration over a 12- to 15-year period of time. Our product candidate AMT-130 consists of an AAV5 vector carrying an artificial micro-RNA specifically tailored to silence the huntingtin gene. The therapeutic goal is to inhibit the production of the mutant protein (mHTT) that causes the disease. Using AAV vectors to deliver micro-RNAs directly to the brain represents a highly innovative approach to treating Huntington's disease.

Data from multiple preclinical studies across five animal models show that a single administration of AMT-130 resulted in a dose-dependent and sustained reduction of mutant huntingtin protein in both the deep structures of the brain. We are encouraged by the significant reductions in mutant huntingtin protein, and believe that knock-down of this magnitude has the potential to significantly alter the course of the disease. The FDA has completed its review of our Investigational New Drug (IND) application for AMT-130, allowing us to begin our planned Phase I/II study. We expect that this year AMT-130 will become the first one-time administered gene therapy to enter clinical testing for the treatment of Huntington's disease.

Preclinical Targets
As we focus on advancing our first two potentially transformative gene therapies for hemophilia B and Huntington's disease, we are looking farther out on the horizon for new, life-changing applications of our expertise and technologies. We currently are exploring additional gene therapy targets that include hemophilia A, Fabry disease, Spinocerebellar ataxia type 3 (SCA3), and – through our research collaboration with Bristol-Myers Squibb – cardiovascular disease.