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. Through recent collaborations and our strategic partnership with Bristol-Myers Squibb to develop gene therapies for cardiovascular diseases, we have taken the next steps toward developing gene therapies targeting chronic and degenerative diseases that affect larger populations.

uniQure has built the world's leading gene therapy platform.

Our gene therapy technology consists of three key elements:

1. A therapeutic gene cassette that carries a transgene that encodes for the expression of a therapeutic protein.

2. A best-in-class AAV-based vector delivery system for delivering the gene cassette. We use the AAV5 variant, or serotype, of vector in our product candidates in hemophilia B and Huntington's disease, and have exclusive, worldwide rights to AAV5 for use in therapeutic products delivered to the brain or liver.

3. Administration technologies to effectively deliver the relevant transgene into the liver or central nervous system.

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 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 Padua variant of Factor IX (FIX-Padua).

Clinical data from our Phase I/II trial demonstrate that our AAV5-based gene therapy has been safe, effective and durable, with no loss of efficacy at up to two and a half years of observation. No patient in the study has had any loss of Factor IX (FIX) activity or capsid-specific, T-cell-mediated immune response.

We believe AMT-061 has the potential to provide lower immunogenicity and durable, consistent and curative benefits to nearly all patients with hemophilia B. In November, uniQure announced initial clinical data in patients treated with AMT-061. These data showed that therapeutic levels of Factor IX (FIX) activity were achieved and sustained in three patients at six weeks after a single administration of AMT-061, with mean FIX activity for the three patients of 31% -- exceeding threshold FIX levels generally considered sufficient to significantly reduce the risk of bleeding events. uniQure is currently enrolling patients in the Phase III HOPE-B pivotal study, with dosing expected to begin in the first quarter of 2019.

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 very 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. We expect to file an Investigational New Drug (IND) application in 2018 and to become the first AAV gene therapy to enter clinical development for 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.