uniQure

Corporate Overview

Founded: 1998 in the Netherlands as Amsterdam Molecular Therapeutics (AMT)
Listed on NASDAQ: February 5, 2014 (QURE)
Full-time employees: 200 (~120 in Amsterdam, the Netherlands, ~80 in Lexington, Mass.)
Chief Executive Officer: Matt 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

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, Mass. 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.

Three Key Areas of Focus, each with proof-of-concept

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). FIX-Padua expresses a protein that has been reported to provide an approximate eight to nine-fold increase in FIX activity compared to the wild-type FIX protein.

We believe AMT-061 has the potential to provide lower immunogenicity and durable, consistent and curative benefits to all, or nearly all, patients with severe and moderately severe hemophilia B. uniQure plans to expeditiously advance AMT-061 into a pivotal study in 2018.

We presented updated clinical data from our Phase I/II trial in December 2017. The data demonstrate that our AAV5-based gene therapy has been safe, effective and durable, with no loss of efficacy at up to two 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.

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 which silences the mutant huntingtin gene that causes the disease. The therapeutic goal is to inhibit the production of the mutant protein. Using AAV vectors to deliver micro-RNAs directly to the brain represents a highly innovative approach to treating Huntington's disease.

In April 2017, uniQure published preclinical studies that support moving forward with clinical trials of this promising gene therapy, including data demonstrating widespread transduction in the central nervous system following direct injection of uniQure's AAV5 vector in a large animal model, as well as data from a second study demonstrating widespread and effective AAV5 vector distribution and extensive silencing of the human mutant huntingtin gene (HTT) in a large animal model. We expect to file an Investigational New Drug (IND) application and enter clinical development for AMT-130 in 2018.

Cardiovascular Disease

In April 2015, uniQure signed a strategic partnership with Bristol-Myers Squibb to discover and develop novel gene therapies to treat a range of cardiovascular diseases, initially focused on the one-time treatment of congestive heart failure (CHF) by selectively restoring cardiac deficiency of the calcium-binding protein S100A1, a master regulator of myocardial function. AAV-S100A1 has shown long-term therapeutic efficacy, safety and reduced mortality in a human-relevant, in vivo heart failure model compatible with clinical drug regimens. uniQure and Bristol-Myers Squibb continue to conduct IND-enabling studies for S100A1. In addition to S100A1, uniQure and Bristol-Myers Squibb have initiated work on three additional targets designated under the collaboration.