

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.

