BioSpectrum ASIA EDITION
the business of Bio & Health Sciences

multiQure begins manufacturing phase for MQ-201 Huntingtonâ??s disease gene therapy and selects Andelyn Biosciences as manufacturing partner

November 27, 2026 | News

The US contract manufacturer will develop the production process for MQ-201 and support the manufacture of clinical material for its planned first administration to patients

multiQure has entered the manufacturing phase for MQ-201, its investigational RNA interference (RNAi) gene therapy for Huntington's disease. The Polish biotechnology company has selected Andelyn Biosciences of Columbus, Ohio, as its manufacturing partner.

Andelyn will be responsible for manufacturing the plasmids and the adeno-associated virus serotype 5 (AAV5) vector under good manufacturing practice (GMP) for planned clinical use. The companies have signed an agreement for the first stage of work and are finalising a broader framework agreement.

The selection marks a step towards the first administration of MQ-201 to patients. Viral vector manufacturing is among the longest, most complex and most expensive stages of gene therapy development, and it can shape when a programme is ready to enter the clinic.

Manufacturing development and clinical supply

The first stage will use Andelyn's AAV Curator platform to develop and scale the manufacturing process for MQ-201. Work will include developing a vector titre assay tailored to multiQure's expression cassette, preparing plasmids and optimising cell culture and purification. The stage will culminate in the production of a research-grade AAV5 vector batch in a 50 L bioreactor.

The companies plan to use the same manufacturing platform, based on suspension HEK293 cell culture, for process development and GMP clinical production. According to multiQure, this approach avoids the need to transfer the process to another platform and reduces risk as the programme moves towards clinical manufacturing.

multiQure has committed to engaging Andelyn for subsequent work orders leading to the manufacture of GMP clinical material. The parties have agreed on the scope of work, timeline and budget, and are finalising the framework agreement.

“Selecting a manufacturer changes the nature of our work: from now on, we are preparing a therapy that we intend to administer to the first patients with Huntington's disease. The viral vector is the most complex and costly part of the path to the clinic, so we were looking for a partner that has already produced hundreds of clinical batches and whose facility has passed an FDA inspection and EU audits. After comparing several leading manufacturers, we chose Andelyn. We are starting right away, in parallel with finalizing the framework agreement, because in gene therapy it is manufacturing that sets the pace of the entire program,” said SÅ?awomir Wójcik, Member of the Management Board of multiQure.

Andelyn is a contract development and manufacturing organisation specialising in viral vectors for gene therapies. The company said it has more than 20 years of experience and has produced more than 500 cGMP batches of clinical material for over 80 clinical trials worldwide. Its Columbus facility has passed a US Food and Drug Administration (FDA) pre-licence inspection and audits by EU Qualified Persons.

“We are very pleased that multiQure has chosen Andelyn as its manufacturing partner for MQ-201. Selecting a vector manufacturer is one of the most important decisions in gene therapy development, and for multiQure it marks the start of the decisive phase of preparations for the first administration of the therapy to patients. There is still no approved treatment that slows the progression of Huntington's disease, which is why we are glad to bring our expertise and join this promising program that aims to silence the mutant copy of the HTT gene,” said Matt Niloff, Chief Commercial Officer of Andelyn Biosciences.

Clinical and regulatory preparations

With Andelyn on board, multiQure said it has identified partners across the areas needed for the planned first administration of MQ-201. A team of consultants and advisers assembled in September 2026 oversees manufacturing. Its members include people with previous experience working with uniQure.

On the clinical side, multiQure has signed a letter of intent with Bródno Masovian Hospital and Professor MirosÅ?aw ZÄ bek of the Interventional Neurotherapy Center regarding experimental use of the therapy. Munich-based Granzer Regulatory Consulting & Services has supported the programme’s regulatory preparations since October, including work on the investigational new drug (IND) application.

In its announcement, multiQure said the FDA had accepted uniQure’s accelerated approval application for AMT-130 for priority review, with a target action date of 1 May 2027. Both AMT-130 and MQ-201 use an AAV5 vector to target the HTT gene. According to multiQure, MQ-201 is designed to silence only the mutant copy, leaving the normal copy intact.