

FOR IMMEDIATE RELEASE

SK pharmteco and Genethon Showcase French Manufacturing Strength Behind Pivotal DMD Gene Therapy Program

Ongoing support for GNT-0004 highlights the capability, readiness, and continued momentum of SK pharmteco's viral vector operations in France as part of its global network

Rancho Cordova, CA (July 21, 2026) — [SK pharmteco](#) today announced details of its ongoing strategic manufacturing partnership with [Genethon](#), a France-based pioneering nonprofit research and development organization dedicated to gene therapy for rare diseases.

Through its viral vector manufacturing operations in France, Yposkesi, an SK pharmteco company, has supported the development and manufacturing of Genethon's GNT-0004, an investigational AAV8-micro-dystrophin gene therapy for Duchenne Muscular Dystrophy (DMD).

The collaboration reflects not only continued progress for GNT-0004 but also the strength of SK pharmteco's French viral vector capabilities within its broader global manufacturing network. [With newly added commercial-scale infrastructure now operational in France](#) and complementary capacity in the United States, SK pharmteco is positioned to support customers from pivotal clinical supply through future commercial production.

The collaboration has delivered strong manufacturing scale results and met rigorous quality benchmarks to support the program's clinical advancement. To date, the collaboration has yielded:

- **Twenty (20) Batches of AAV8 Micro-dystrophin:** Manufactured, tested, and released at a 400 L scale (including 18 current Good Manufacturing Practice [cGMP] batches).
- **Master and Working Cell Banks (MCB/WCB):** Successful manufacture, testing, and release to ensure long-term production stability.
- **Analytical & Stability Foundation:** Completed robust stability studies and full validation of analytical methods.

These critical chemistry, manufacturing, and controls (CMC) activities provided the regulatory foundation for Genethon to advance through Phase I/II trials, and supported [the launch of the pivotal Phase III clinical trial in Europe and the UK, which was authorized in 2025 by the European Medicines Agency \(EMA\) and the UK Medicines and Healthcare products Regulatory Agency \(MHRA\)](#).

This ongoing randomized, placebo-controlled, double-blind, multicenter pivotal phase is expected to enroll a total of 72 ambulatory boys aged 6 to 10 with DMD, and the selected dose is lower than that used for other DMD gene therapy drug candidates in clinical trials or approved for Duchenne muscular dystrophy.



Compelling long-term data unveiled at the 2026 American Society of Gene & Cell Therapy (ASGCT) Annual Meeting, demonstrated sustained efficacy at two years, meaningful and lasting improvements in muscle function, and a favorable safety profile in the first patients treated with GNT-0004.

SK pharmteco is currently manufacturing additional clinical batches to directly support this pivotal trial, as well as documentation and readiness activities to support future process validation and broader commercial manufacturing preparedness.

This collaboration also underscores the [continued momentum of SK pharmteco's viral vector manufacturing operations in France](#). By supporting a complex AAV program through GMP manufacturing, analytical validation, and pivotal-trial supply, SK pharmteco demonstrates that its French site is active, experienced, and well-positioned to support additional late-stage viral vector programs in Europe and beyond.

"Our partnership with Genethon embodies SK pharmteco's core mission: translating complex scientific innovation into reliable, scalable therapies," said Joerg Ahlgrimm, CEO at SK pharmteco. "This program demonstrates our ability to support complex AAV manufacturing not only through pivotal clinical supply, but also through the quality, technical, and operational readiness required for future commercial manufacturing. It also reinforces the strength of our viral vector capabilities in France as part of the broader SK pharmteco Viral Vector organization."

"Bringing a gene therapy from the lab to a pivotal Phase III trial requires an exceptionally high standard of manufacturing excellence and regulatory compliance," said Frederic Revah, CEO at Genethon. "The technical expertise and dedication demonstrated by the SK pharmteco team in France have been instrumental in successfully reaching this clinical milestone. We look forward to building on this momentum as we continue advancing GNT0004."

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About SK pharmteco

SK pharmteco is a global contract development and manufacturing organization (CDMO) with production sites, research & development facilities, and analytical laboratories across the U.S., Europe, and South Korea. The company's core capabilities center on small molecules, peptides, and viral vectors, providing the specialized expertise needed to bring complex therapies to market. Through these pillars, SK pharmteco supports biopharmaceutical partners of all sizes with comprehensive development and manufacturing solutions worldwide. SK pharmteco is a subsidiary of SK Inc. (KRX: 034730) (SK), the strategic investment company for SK Group, South Korea's second-largest conglomerate.

About Duchenne muscular dystrophy

Duchenne muscular dystrophy is a rare progressive genetic disease that affects all the muscles in the body and mainly boys (1 in 5,000). It is caused by abnormalities in the gene responsible for the production of dystrophin, a structural protein essential for the stability of muscle fiber membranes and their metabolism. The absence of dystrophin leads to progressive degeneration of the skeletal

and cardiac muscles, loss of walking and respiratory abilities, cardiomyopathy, and death, usually between the ages of 20 and 40.

About GNT0004 and the trial

The gene therapy product GNT0004 consists of an AAV8 (adeno-associated virus) vector and the optimized hMD1 transgene, a shortened but functional version of the gene encoding dystrophin, the protein that is deficient in people with Duchenne muscular dystrophy. This vector is designed to express itself in muscle tissue and also in the heart, thanks to a Spc5-12 promoter sequence specific to these tissues. GNT0004 is administered by a single intravenous injection. It was developed by Genethon, in collaboration, for the preclinical phases, with the teams of Prof. Dickson (University of London, Royal Holloway), the Institute of Myology (Paris) and Caroline Le Guiner (INSERM/University of Nantes/Nantes University Hospital).

About Genethon

A pioneer in the discovery and development of gene therapies for rare diseases, Genethon is a non-profit laboratory created by the AFM-Telethon. The first gene therapy drug, to which Genethon contributed, has been approved for marketing for spinal muscular atrophy. With more than 240 scientists and experts, Genethon's goal is to develop innovative therapies that change the lives of patients suffering from rare genetic diseases. Fifteen gene therapy products resulting from Genethon's research, or to which Genethon has contributed, are currently undergoing clinical trials for diseases of the liver, blood, immune system, muscles, and eyes. Others are preparing for clinical trials over the next five years. www.genethon.com

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