Yposkesi selected to produce clinical and large-scale commercial material of micro-dystrophin for Genethon and Sarepta Therapeutics program

Its capacity to produce AAV (Adeno Associated Virus) materials at large-scale will support Genethon and Sarepta's project to take micro-dystrophin to clinical trials this year

Corbeil-Essonnes, France, January 9, 2020 – Yposkesi, a leading full-service Contract Development and Manufacturing Organization (CDMO) for preferred access and reserved capacity for cGMP grade viral vector production, today announces that it has been selected to produce clinical and large-scale commercial AAV micro-dystrophin material within the co-development program between Genethon and Sarepta Therapeutics focused on Duchenne muscular dystrophy.

Genethon and Sarepta recently announced they are extending their collaboration on developing an innovative gene therapy for Duchenne muscular dystrophy, which has demonstrated significant efficacy in pre-clinical testing. Yposkesi's selection to produce clinical and large-scale commercial batches is based on its innovative proprietary suspension production process using a distinct producer clone and a specific transfecting agent.

Yposkesi is currently doubling the size of its production facilities to increase volume capacity of its bioreactors, enabling it to meet its clients' growing needs. With the construction of an additional 5,000m^2 site underway, Yposkesi plans to deliver commercial batches in 2022/2023.

"Yposkesi is proud to have been selected to produce and deliver AAV material at large-scale using its proprietary suspension-based process for Duchenne muscular dystrophy, a neuromuscular disease that requires high doses," said Alain Lamproye, CEO of Yposkesi. "The significant investments made in honing our expertise, expanding our facilities, as well as developing innovations to increase production capacity and process efficiency are aimed at serving gene therapy developers, especially those currently facing a bottleneck in manufacturing."

About Duchenne muscular dystrophy

Duchenne muscular dystrophy is a rare, progressive genetic disease that affects all the muscles of the body in approximately one in 3,500 boys. It is the most common neuromuscular disease in children. It is linked to abnormalities in the DMD gene, which is responsible for the production of dystrophin, a protein that is essential for the proper functioning of the muscles. This gene has the characteristic of being one of the largest in our genome (2.3 million base pairs of which more than 11,000 are coding). Because of this size, it is technically impossible to insert the complete DNA of dystrophin in a viral vector (or even the only 11,000 coding base pairs), as is usual in gene therapy.

About Yposkesi

Yposkesi is a leading Contract Development & Manufacturing Organization (CDMO) for gene therapy vector manufacturing. Created in November 2016 in Corbeil-Essonnes (France) as a spin-off from the world-class gene therapy pioneer Genethon, Yposkesi provides integrated services covering bioprocess development (USP & DSP) from small/pilot to large-scale production, analytical development, GMP manufacturing of lentiviral and AAV vectors and regulatory support. Its current facility consists of a 50,000ft^2 (approx. 5,000m^2) building, operating multiple manufacturing suites for bulk drug substance and Fill&Finish. By 2022/23, Yposkesi will increase its global footprint to 100,000ft^2 (approx. 10,000m^2) with a second large-scale facility designed for EMA and FDA compliance. Capitalizing on the more than 25 years' expertise of Genethon, Yposkesi invests significantly in innovation in bioprocessing to deliver on high-quality projects, cost-effectively. Yposkesi has also entered into several strategic partnerships including those with Axovant Gene Therapies, Servier and Orchard Therapeutics.

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