Yposkesi's clinical batches of G1XCGD lentiviral vector used in rare immune system disease study

International research team published initial clinical trial results in Nature Medicine of nine X-Linked Chronic Granulomatous Disease (CGD) patients

Corbeil-Essonnes, France, January 29, 2019 – Yposkesi, a leading Contract Development and Manufacturing Organization (CDMO) for preferred access and reserved capacity for cGMP grade viral vector production, today announces it produced the lentiviral vectors used in the gene therapy clinical trial in patients severely affected with X-Linked chronic granulomatous disease, a rare inherited disease of phagocytic cells. The primary objectives of the study were to assess the safety and evaluate the efficacy and stability of biochemical and functional reconstitution in the progeny of engrafted cells at 12 months. These were met in six of the nine patients, suggesting that autologous gene therapy is a promising approach for CGD patients, according to the report. The work on X-Linked chronic granulomatous disease patients is published in Nature Medicine.

"Yposkesi is very pleased to have supplied the high quality viral vectors for this clinical study of patients with X-Linked chronic granulomatous disease," said Alain Lamproye, CEO of Yposkesi. "It is an exciting development for this treatment that uses the patient's own stem cells rather than those of a donor."

Chronic granulomatous disease is characterized by a genetic mutation in one of five genes that help white blood cells attack and destroy bacteria and fungus using a chemical mechanism. CGD patients who lack this defensive chemical mechanism are more prone than most people to severe or life-threatening infections. The most common form of CGD is a subtype called X-CGD, which affects only males and is caused by a mutation in a gene found on the X-chromosome. Yposkesi was selected to contribute to the clinical trial because of its extensive expertise and track record in the manufacturing of lentiviral vectors, which it developed under Genethon, a pioneer in gene therapy.

Dr. Donald Kohn, a member of the Eli and Edythe Broad Center for Regenerative Medicine and Stem Cell Research at UCLA, led the research, which was supported by grants from the California Institute for Regenerative Medicine; the National Heart, Lung and Blood Institute and the National Institute of Allergy and Infectious Diseases, both at the National Institutes of Health; the Wellcome Trust; Boston Children's Hospital; the National Institute for Health Research Great Ormond Street Hospital Biomedical Research Centre; the Institute for Health Research Biomedical Research Centre at University College London Hospitals NHS Foundation Trust and University College London; the Great Ormond Street Hospital Children's Charity; the AFM-Téléthon, French Muscular Dystrophy Association and the European Commission through the Net4CGD consortium.

About Yposkesi

Yposkesi is a leading Contract Development & Manufacturing Organization (CDMO) for gene therapy vector manufacturing. Created in November 2016 in Corbeil-Essonnes (France) by AFM-Telethon and the SPI fund managed by Bpifrance,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, 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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