# Yposkesi's viral vectors used in gene therapy-based clinical trial

## EuroFanColen researchers published in Nature Medicine first clinical results for Fanconi anemia patients

**Corbeil-Essonnes, France, September 9, 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 viral vectors used in the gene therapy-based clinical trial in patients with Fanconi anemia. In this study, the authors demonstrate for the first time the production of blood cells derived from the patients' genetically corrected stem cells. The work on Fanconi anemia patients is published in Nature Medicine: https://www.nature.com/articles/s41591-019-0550-z

"Yposkesi is very pleased to have contributed to this major study of patients with Fanconi anemia," said Alain Lamproye, CEO of Yposkesi. "The results obtained in this study offer new opportunities in the treatment of Fanconi anemia patients, while also demonstrating the quality and efficiency of our bioproduction processes."

Fanconi anemia is a rare genetic disease characterized by bone marrow failure and cancer predisposition that appears in most patients with poor blood cell production at very young ages. Yposkesi was selected to contribute to the clinical trial because of its extensive expertise and track record in manufacturing of lentiviral vectors, which it developed under Genethon, a pioneer in gene therapy.

Yposkesi supplied the viral vectors to the EuroFancoLen program, which carried out the research. Coordinated under Dr. Juan Bueren of the Center for Energy, Environment and Technology (CIEMAT), it involved the Center for Biomedical Network Research on Rare Diseases (CIBERER) and the Institute of Health Research of the Jiménez Díaz Foundation (IIS-FJD). It was guided under the clinical direction of Dr. Julián Sevilla of the Foundation of the Hospital del Niño Jesús, in Madrid (Spain), the clinical sponsor of the trial. The first authors of the study are Dr. Paula Río and Dr. Susana Navarro, affiliates of the CIEMAT / CIBERER / IIS-FJD Consortium.
More information on EuroFancoLen

**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 50,000 $ft^2$ (approx. 5,000 $m^2$) building, operating multiple manufacturing suites for bulk drug substance and fill&finish. By 2021 it will have increased its global footprint to 100,000 $ft^2$ (approx. 10,000$m^2$) 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.
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