



FOR IMMEDIATE RELEASE

SK pharmteco Cell & Gene Europe, AP-HP, AFM-Téléthon and Institut *Imagine* Announce Collaboration for Lentiviral Vector Production for an Innovative Sickle Cell Disease Clinical Trial

RANCHO CORDOVA, CA / PARIS, FRANCE (March 4, 2025) [SK pharmteco Cell & Gene Europe](#), a global contract development, manufacturing, and analytical testing organization serving the pharmaceutical and cell & gene therapy industries, along with [Assistance Publique – Hôpitaux de Paris \(AP-HP\)](#), and [Institut Imagine \(Inserm, AP-HP, Université Paris Cité\)](#) announced the signing of a contract for the production of a 200L CGMP clinical batch of lentiviral vector (LVV) and associated regulatory support. AFM-Téléthon is the main funder of the final preclinical phases and the technical production of the clinical batches, enabling the teams to reach this new milestone.

This collaboration focuses on developing a gene therapy against sickle cell disease using an *ex vivo* hematopoietic stem and progenitor cell (HSPC) gene modification approach with LVV. This innovative approach is based on a newly designed vector by the laboratories of Annarita Miccio and Marina Cavazzana, research director at Institut *Imagine*, and on previous collaborative work with Mario Amendola at Généthon laboratory.

The project leverages SK pharmteco's LentiSure™ platform and transgene plasmid, with analytical testing conducted at its facility in France. It is a key milestone towards starting a clinical trial supported and sponsored by AP-HP and translating Institut *Imagine*'s research to the clinical stage.

“We are thrilled to partner with AP-HP and Institut *Imagine* on this critical project,” said Patrick Mahieux, President of SK pharmteco Cell & Gene Europe. “The successful production of both the technical and clinical batches will be yet another demonstration of the power of our LentiSure™ platform and our commitment to advancing gene therapies for serious diseases like sickle cell disease.”

“We are proud to support this key validation phase for this gene therapy drug candidate for sickle cell disease, a rare and extremely serious blood disorder that affects thousands of people in France and millions worldwide, and to enable the researchers we have supported for many

years, thanks in particular to the Telethon, to take the final step towards treating these patients” said Laurence Tiennot-Herment, President of AFM-Téléthon

“This collaboration represents a significant step forward in our efforts to develop a safe and effective gene therapy for sickle cell disease,” said Dr Jean-Roch Fabreguettes, clinical researcher and coordinator of AP-HP’s MEARY center for cell and gene therapy and manufacturing of advanced therapy medicinal products. “We are confident that this partnership with SK pharmteco and Institut *Imagine* will bring us closer to improving patients’ lives.”

“Institut *Imagine* is dedicated to translating cutting-edge research into innovative therapies,” added Pr Marina Cavazzana, Head of Research. “This partnership with SK pharmteco and AP-HP enables us to move our pre-clinical findings into the clinic, offering hope for patients with sickle cell disease.”

Sickle cell disease, a genetic disease that affects red blood cells, is most often diagnosed at birth. The most frequent symptoms are anemia, increased infection susceptibility, and painful attacks affecting various organs. According to the World Health Organization, approximately five million people worldwide live with sickle cell disease. It is a chronic disease for which there is currently no cure; this clinical trial, therefore, represents a major hope for patients affected by this disease.

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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 Korea. The company partners with biopharmaceutical companies of all sizes to manufacture Active Pharmaceutical Ingredients (API) and intermediates, cell and gene therapy technologies, registered starting materials, and analytical services for the biopharmaceutical industry 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.

<https://www.skpharmteco.com/>

About AP-HP

AP-HP is a university hospital center with a European dimension and recognized worldwide. It is

organized into six university hospital groups (AP-HP. Center - University Paris Cité; AP-HP. Sorbonne University; AP-HP. North - University Paris Cité; AP-HP. University Paris Saclay; AP-HP. Henri Mondor University Hospitals and AP-HP. Paris Seine-Saint-Denis University Hospitals) and is structured around five universities in the Paris region. Its 38 hospitals welcome 8.3 million sick people each year: for consultations, emergencies, scheduled hospitalizations or home hospitalization. It provides a public health service for all, 24 hours a day, and this is both a duty and a source of pride for it. AP-HP is the largest employer in the Ile de France region: 100,000 people – doctors, researchers, paramedics, administrative staff and workers – work there.



<http://www.aphp.fr>

About AFM-Téléthon

The French Muscular Dystrophy Association (AFM) federates patients with neuromuscular diseases and their families. Thanks to donations from France's annual Telethon, the AFM-Telethon has become a major player in biomedical research for rare diseases in France and worldwide. It currently funds about 350 research programs and 40 clinical trials in different genetic diseases affecting the eye, blood, brain, immune system, motor neurons and muscles... The AFM-Telethon has also created three research and development institutes dedicated to gene therapy, stem cells and myology.

To learn more visit www.afm-telethon.fr/en

About Institut *Imagine*

Located on the campus of the Necker-Enfants Malades Hospital, Institut *Imagine* is a European leader in research, care, and teaching on genetic diseases. Its unique architecture, designed by Jean Nouvel and Bernard Valéro, brings together 1,000 researchers, physicians, teacher-researchers, engineers and healthcare personnel in a single location to work with patients, with the ambition of accelerating research and diagnosis and therapeutic innovation to change the lives of families affected by genetic diseases. Institut Imagine has been certified “Institut hospitalo-universitaire” (IHU), in 2011 and 2019 and “Institut Carnot” in 2020. It is supported by six founding members, including AP-HP, Inserm and Université Paris Cité, as well as by private partners and patrons. Every day in France, 64 babies are born with a genetic disease. Nearly 8,000 genetic diseases affect more than 3 million people, of which nearly one in two is

undiagnosed and more than 8 in 10 have no dedicated treatment. Faced with this public health emergency, the challenge is twofold: to diagnose and to cure.

www.institutimagine.org

Contact:

Keith Bowermaster, APR, CCMP

Communications Consultant

keith.bowermaster@skpt.com

Website: www.skpharmteco.com

SOURCE: SK pharmteco

Agence Havas RED

Juliette Arcos - imagine.presse@havas.com

Institut *Imagine*

Anne Lenoir - anne.lenoir@institutimagine.org

AFM-Téléthon

Stéphanie Bardon – sbardon@afm-telethon.fr / 06.79.34.15.68