

Press release

Genethon and Eukarÿs announce a strategic partnership to develop a breakthrough technology to reduce the biomanufacturing cost of gene therapies

Evry, January 23, 2025- Genethon, a pioneer and leader in the research and development of gene therapies for rare diseases, and Eukarÿs, a biotechnology company developing a disruptive technology that significantly increases biomanufacturing yields, today announced the signing of a strategic partnership. The collaboration aims to reduce the production costs of gene therapy medicines by harnessing Eukarÿs' proprietary C3P3 technology, an innovative messenger RNA (mRNA) expression system in mammalian cells.

Gene therapies offer immense hope for patients suffering from rare and common diseases. However, the biomanufacturing of these therapies remains complex and costly, representing a major obstacle to their development and commercialization. Improving production technologies and dramatically increasing biomanufacturing yields are therefore strategic goals to improve patient access to these cutting-edge therapies.

Eukarÿs has developed an engineered enzyme, C3P3, which significantly increases the production capacity of cells used for biopharmaceutical synthesis, by enhancing their mRNA synthesis capabilities. Combined with Genethon's expertise and technologies, this innovative approach could significantly improve the production of AAV (Adeno-Associated Virus) vectors, one of the main vector types used to deliver therapeutic genes to patients.

As part of this collaboration, Genethon will contribute its expertise in AAV vector design and production, as well as its technologies and biomanufacturing facilities, while Eukarÿs will provide its patented C3P3 technology and deep expertise in mRNA synthesis.

Patrick Santambien, Director of Technological Development at Genethon - "For more than fifteen years, Genethon has been pushing the boundaries of gene therapy biomanufacturing by focusing on innovative approaches. Eukarÿs' technology is truly disruptive to biomanufacturing in general and holds great promise for delivering gene therapy products based on AAV vectors under optimal conditions, while drastically reducing biomanufacturing costs. We are excited about this partnership, which could pave the way for much more affordable therapies."

Guillaume Prunier, CEO of Eukarÿs - "We are very pleased and proud to partner with Genethon. Our enzyme, which is now commercially available, is universal and requires no adaptation, with two exceptions: lentiviruses and AAV. Given the importance of gene therapy for patients with serious diseases and the industrial challenge it represents, we are delighted to be working with Genethon, a leader in this

field, to accelerate this adaptation. We are confident that the outcome of this partnership will be a game changer for both industry and patients".

About the C3P3 Enzyme

The C3P3 synthetic enzyme is the fruit of 14 years of development at Eukarys and is protected by three international patent families. This universal technology enables a 5 to 7-fold increase in production yields in all mammalian cell types. It can be seamlessly integrated into any existing industrial processes without additional investment or modification and complements all current biomanufacturing technologies. C3P3 is suitable to produce virtually all biopharmaceuticals, including monoclonal antibodies, recombinant proteins and Virus-like particles (VLPs).

About Genethon

A pioneer in the discovery and development of gene therapies for rare diseases, Genethon is a non-profit organization created by the AFM-Téléthon. The first gene therapy to treat spinal muscular atrophy, incorporating technologies developed at Genethon, is marketed worldwide. With over 200 scientists and professionals, Genethon pursues its goal of developing innovative therapies that change the lives of patients suffering from rare genetic diseases. Thirteen products from Genethon's R&D or collaborations are in clinical trials for diseases of the liver, blood, immune system, muscles and eyes. A further seven products could enter clinical trials in the next five years.

To find out more: www.genethon.com

About Eukarys

Eukarys is a biotechnology company that has developed the C3P3 breakthrough technology with the aim of transforming the production of all biopharmaceuticals and making them more accessible.

Founded in 2010 as a private company and based at the Génomipole incubator in Evry, Eukarys was awarded a Sanofi *Golden Ticket* in 2024, becoming the first winner in the biomanufacturing sector. The company also won the prestigious *Prix Galien USA* in 2019 (MedStartup category). Eukarys is supported by the French government and Bpifrance through the France 2030 DeepTech program and has twice won the i-Lab competition organized by Bpifrance.

To find out more: www.eukarys.com

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