Press release
January 14, 2026

Genethon Signs Licensing Agreement with AskBio for the Development of an Investigational Gene Therapy for Pompe Disease

PARIS, France (January 14, 2026) - Genethon, a leading laboratory in gene therapy for rare diseases, today announced that it has entered into an exclusive, worldwide licensing agreement with AskBio, a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, for the use of a patented component of AB-1009 for the treatment of Pompe disease.

Pompe disease is a rare genetic disorder caused by a deficiency in the GAA enzyme, leading to glycogen accumulation in cells, particularly in muscle tissue.

The 'Immunology and Liver Diseases' team at Genethon, led by Giuseppe Ronzitti, developed and demonstrated the preclinical efficacy of a gene therapy approach using a transgene encoding a truncated form of GAA. In animal studies, this approach corrected glycogen accumulation in muscle and the central nervous system and improved cardiac hypertrophy as well as muscular and respiratory dysfunction*. This component of AB-1009, protected by patents held by Genethon and other French institutions, is now licensed to AskBio for the development of a gene therapy product to treat Pompe disease.

This agreement illustrates Genethon's ability to transform its scientific advances into therapeutic opportunities for patients with rare diseases by collaborating with industry partners to advance clinical development.

"Genethon is delighted with this agreement with AskBio, which enables the development of a gene therapy for patients suffering from this particularly severe condition," said Frédéric Revah, CEO of Genethon. "This agreement demonstrates the quality of Genethon's research and its ability to design innovative treatments, driven by its mission to provide therapeutic solutions to patients with rare diseases."

Askbio anticipates recruiting its first patient in early 2026.

* Rescue of Pompe disease in mice by AAV-mediated liver delivery of secretable acid α-glucosidase. Puzzo F, Colella P, Biferi MG, Bali D, Paulk NK, Vidal P, Collaud F, Simon-Sola M, Charles S, Hardet R, Leborgne C, Meliani A, Cohen-Tannoudji M, Astord S, Gjata B, Sellier P, van Wittenberghe L, Vignaud A, Boisgerault F, Barkats M, Laforet P, Kay MA, Koeberl DD, Ronzitti G, Mingozzi F.
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About Pompe disease

Pompe disease is a hereditary genetic disorder linked to a deficiency in acid alpha-glucosidase (GAA), which leads to the accumulation of glycogen in lysosomes, causing cellular damage in various tissues, particularly in the heart, muscles, and nervous system. In infants with severe forms of the disease, the heart muscle is often affected, which is the main cause of death. In patients with late-onset forms, the respiratory system and mobility are generally the most affected by the disease, often requiring the use of a wheelchair and respiratory assistance, and potentially shortening life expectancy.

About Genethon

A pioneer in the discovery and development of gene therapies for rare diseases, Genethon is a non-profit laboratory created by the AFM-Telethon. The first gene therapy drug, to which Genethon contributed, has been approved for marketing for spinal muscular atrophy. With more than 240 scientists and professionals, Genethon's goal is to develop innovative therapies that change the lives of patients suffering from rare genetic diseases. Thirteen gene therapy products resulting from Genethon's research, or to which Genethon has contributed, are currently undergoing clinical trials for diseases of the liver, blood, immune system, muscles, and eyes. Others are in preparation for clinical trials over the next five years.

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