



Press release
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Genethon and Ampersand Biomedicines announce partnership to design novel AAV vectors with enhanced tissue specificity for more effective and better tolerated gene therapies

Genethon, the French pioneer in the discovery and development of gene therapies for rare diseases, and Ampersand Biomedicines, a Flagship Pioneering multi-product platform company developing smarter medicines that act specifically at the site of disease and nowhere else, announce an exclusive collaboration to engineer a new generation of AAV capsids with superior tissue specificity. This strategic partnership brings together two highly complementary R&D teams with synergistic scientific capabilities, aiming to unlock the full therapeutic potential of AAV-based gene therapy.

Adeno-associated virus (AAV) vectors are widely used in gene therapy due to their strong therapeutic performance. However, natural capsids display a pronounced tropism for the liver, often requiring high vector doses to deliver sufficient therapeutic effect to other target tissues. Requiring high doses has impacts on safety, tolerability, and cost.

To overcome these limitations, Genethon and Ampersand will jointly design, test, and advance novel AAV vectors capable of reaching target tissues with much greater precision.

"Precision targeting is a key driver for improving both the efficacy and tolerability of gene therapy. By directing the vector exclusively to the tissues of interest, higher potency with lower doses can be used, systemic exposure is reduced, and immune responses can be minimized. We are delighted with this collaboration with Ampersand Biomedicines, which combines cutting-edge AI-powered drug design technology with Genethon's expertise in gene therapy, and opens up new perspectives for novel generation AAV-gene therapies," emphasizes Frédéric Revah PhD., Chief Executive Officer of Genethon.

"At Ampersand, we aim to address one of medicine's central challenges: delivering therapies exactly where they are needed," said Jason Gardner, D.Phil., Chief Executive Officer and President of Ampersand Biomedicines and CEO-Partner at Flagship Pioneering. *"Since our founding, we have identified exquisitely selective ligands to specific cells across every major tissue and cell type in the human body, enabling a new level of precision in therapeutic targeting. By combining our unique targeting capabilities with Genethon's expertise in AAV engineering, we are looking forward to contributing to the next generation of safer and more efficacious therapies with the potential to transform patient outcomes."*

The partnership leverages Ampersand's cutting-edge Address, Navigate, Determine (AND)[™] Platform, a computationally powered engine coupled with cutting-edge antibody discovery capabilities, to develop a pipeline of smarter medicines that act at the site of disease and nowhere else. Core to the AND[™] Platform is Ampersand's proprietary Address Map, an unprecedented characterization of human biology that identifies optimal addresses for drug localization with cell-specific precision. Under the collaboration, Ampersand will utilize its unique tissue-specific ligands to drive vector specificity. Genethon will integrate such identified localizers onto the surface of AAV capsids to increase the precision targeting of the AAV vectors.

The partnership will initially focus on skeletal muscle, where Genethon is a globally recognized expert, with the intent to expand vector targeting across a broad range of tissues and organs.

By uniting Genethon's world-class gene therapy expertise with Ampersand's leadership in designing and developing precision biologics, this collaboration aims to develop more finely targeted, safer and more effective AAV-based gene therapies.

About Genethon

A pioneer in the discovery and development of gene therapies for rare diseases, Genethon is a non-profit laboratory created by AFM-Téléthon. A first gene therapy drug, to which Genethon contributed, has been approved for spinal muscular atrophy. With more than 240 scientists and experts, Genethon aims to develop innovative therapies that change the lives of patients suffering from rare genetic diseases. Fifteen gene therapy products developed by Genethon, or with its contribution, are currently in clinical trials for liver, blood, immune system, muscle, and eye diseases. www.genethon.com

About Ampersand Biomedicines

Ampersand Biomedicines enables a new way of programming medicines that work precisely where needed in the body and nowhere else. The company's computationally powered Address, Navigate, Determine (AND)[™] Platform identifies ideal addresses for drug localization and informs the design of AND-Body[™] therapeutics that have the optimal therapeutic effect. AND-Body therapeutics combine a localizing antibody that enables cell-specific precision with an actuator specifically chosen for disease modification. This approach enables improved target engagement while sparing on-target, off-tissue side effects. Ampersand Biomedicines was founded by Flagship Labs, the innovation foundry of Flagship Pioneering, in 2021. For more information, please visit www.ampersand.bio, and follow us on [LinkedIn](#).

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