

Sanofi, WhiteLab Genomics, the TaRGeT Laboratory at Nantes University, and Institut *Imagine* launch WIDGeT to make France a global leader in gene therapy.

The WIDGeT consortium (Viral Vector Intelligent Design for Gene Therapy) aims to accelerate the development of gene therapies using viral vectors derived from adenoassociated viruses (AAV) for the treatment of age-related macular degeneration (AMD) and hereditary podocytopathies (kidney diseases), leveraging the potential of artificial intelligence (AI).

Paris, October 23, 2023 - Sanofi, a global healthcare company, WhiteLab Genomics, specializing in AI in genomic medicine, the TaRGeT Laboratory at Nantes University (INSERM UMR 1089), a leading French laboratory in gene therapy, and Institut *Imagine* (AP-HP, Inserm, Université Paris Cité), the premier European center for research, education, and care in genetic diseases, launch the WIDGeT consortium (Viral Vector Intelligent Design for Gene Therapy) to accelerate the development of AAV-based gene therapies for the treatment of rare to common diseases (including kidney and eye diseases) by developing next-generation AAV vectors through artificial intelligence (AI). This consortium is financially supported by the France 2030 plan operated by Bpifrance (Banque publique d'investissement France).

Making France a global leader in AI-based gene therapies

WIDGeT aims to create an innovative ecosystem with excellence in their fields to accelerate the development of new gene therapies with the assistance of AI and innovative administration methods, making France a global leader in this field.

WIDGeT is a winner of the Innovations in biotherapies and bioproduction call for projects under the Biotherapies bioproduction acceleration strategy, one of whose aims is to facilitate the development of healthcare innovations deemed strategic, and biotherapies in particular. It will benefit from a budget of 17.95 million euros operated by Bpifrance. This initiative meets the challenges of the France 2030 plan to unite and mobilize all players in the technologies of the future, in line with the objectives of France BioLead and that of the French government to establish France as the European leader in biopharmaceutical production by 2030.

"We are encouraged by the investment in WIDGeT. As genomic medicine matures, there is an ever-increasing need for optimization of the delivery vehicles for these nucleic acid medicines. Working with WhiteLab Genomics, the TarGeT Lab at Nantes University and Institut Imagine, we aim to create novel adeno-associated viral vectors with tropisms to currently hard-to-reach cells. Targeted vectors will not only create opportunities for new therapeutics, but will lower costs, increase access, and create larger therapeutic windows," added Dr. Christian Mueller, VP Global Head of Genomic Medicine at Sanofi

Maximizing the effectiveness of gene therapies by optimizing viral vectors

Despite the approval of several gene therapy products to treat rare and common diseases, the complexity of using these therapies still faces significant scientific and technical challenges. These treatments rely on vectors used to deliver therapeutic genes into cells, compensating for the mutations responsible for the targeted diseases. Currently, significant technological barriers remain, and there is a need for technological innovation to improve these treatments. Among the biopharmaceutical vectors currently evaluated, Adeno-Associated Viruses (AAV) appear exceptionally promising for gene therapy and in vivo gene transfer, leading to notable progress in the field. The mission of the WIDGeT consortium is to optimize these AAV vectors to enhance the efficiency and specificity of gene therapies, reduce injected doses, minimize undesirable side effects, and help reduce the production costs associated with personalized treatments. The use of innovative AI-based approaches is original in overcoming these difficulties and producing new optimized vectors that are more effective, allowing for reduced product quantities while maximizing therapeutic effectiveness.

"It is a great source of pride for the TaRGeT Laboratory (Nantes University and INSERM) to be part of the WIDGeT public-private consortium. Thanks to the unique combination of complementary and multidisciplinary expertise of the four partners, the consortium offers an innovative approach to developing new gene therapy medicines for major indications in public health. We will contribute our recognized expertise, built over 27 years in the field of AAV vector production and preclinical evaluation. Together, we will transform French gene therapy for the benefit of patients suffering from still incurable eye and kidney diseases," concludes Dr. Oumeya Adjali, Director of the TaRGeT Laboratory.

"Institut Imagine is proud to participate in this large-scale project aiming to integrate AI into the development and optimization of gene therapies. Our expertise in the field of podocytopathies and the use of innovative technological devices will enhance the effectiveness of gene therapies, harnessing the remarkable development of AI in biology and healthcare. These projects once again demonstrate the importance of public-private, multidisciplinary, and multi-partner collaborations, focusing on a rare kidney disease, a well-known model for our teams, to apply the knowledge gained to much more common diseases in the near future," concludes Professor Stanislas Lyonnet, Director of Institut Imagine.

Rapidly developing new optimized vectors with AI

The consortium partners are fully committed to advancing gene therapies with the contributions of AI. Advanced machine learning algorithms developed by WhiteLab Genomics will synergize with TaRGeT's robust bioproduction capabilities, certified by the national biotherapy acceleration strategy. Additionally, TaRGeT and Institut *Imagine* will provide enhanced experimental cellular and physiological qualifications. Sanofi, on its part, will contribute not only its analytical expertise but also its steadfast commitment for the development of the next generation of gene therapy vectors. The use of AI will improve tissue and cellular specificity of AAV variants developed for two targets: podocytes and microglial cells, which are involved in rare kidney diseases (hereditary podocytopathies), with Institut *Imagine*, and neuro-ophthalmic diseases, particularly age-related macular degeneration (AMD), with TaRGeT at Nantes University. Over the next five years, WIDGeT aims to establish technological innovations for vector engineering and improve gene transfer tools to select solutions suitable for rare and common diseases treatable by gene therapy.

"We are delighted to expand our collaboration with Sanofi and initiate partnerships with Institut Imagine and the TaRGeT Laboratory at Nantes University in the development of new genomic medicines. We believe that our technologies have the potential to revolutionize biopharmaceutical development and are confident that this consortium will have a significant impact on patient care. Together, we will turn this vision into reality," declares David Del Bourgo, CEO and co-founder of WhiteLab Genomics.

About Sanofi

We are a global healthcare company, driven by a mission to pursue the miracles of science to improve people's lives. Our teams, present in over a hundred countries, work to transform the practice of medicine to make the impossible possible. We offer therapeutic solutions that can change the lives of patients and vaccines that protect millions of people worldwide, guided by the ambition of sustainable development and our social responsibility. Sanofi is listed on EURONEXT: SAN and NASDAQ: SNY.

About WhiteLab Genomics

Founded in 2019, WhiteLab Genomics is a Deeptech startup developing and operating its AI platform for biotherapies, including gene and cell therapies. Their proprietary technology analyzes complex biological data with artificial intelligence, accelerating the development and reducing the risks associated with those treatments. WhiteLab Genomics collaborates with academic institutions (Genethon,

INSERM, Nantes University) and industrial partners, including Sanofi. WhiteLab Genomics has won numerous awards and is part of French Tech 2030, French Tech Health 20, and was selected by Station F in its first Future 40 selection and is currently hosted and supported in the Future4care incubator resulting from the alliance between Capgemini, Generali, Orange, and Sanofi. It is also a member of the prestigious American accelerator Y-Combinator.

About the TaRGeT Laboratory UMR 1089 (Nantes University INSERM)

The TaRGeT Laboratory is a leading academic research laboratory in translational research applied to gene therapy, recognized for its expertise of over 27 years in the development and preclinical evaluation of gene therapy products using AAV vectors, covering the entire translational chain (genome and viral capsid modification, large-scale bioproduction, efficiency and immunogenicity of AAVs in preclinical models, clinical translation). TaRGeT includes the Center for Vector Production (CPV), one of the 8 integrators in biotherapies and bioproduction labeled by France 2030.

About Institut Imagine

Located on the Necker-Enfants malades hospital campus, Institut *Imagine* is a global leader in research, care, and education on genetic diseases. Its unique architecture, designed by Jean Nouvel and Bernard Valéro, brings together 1,000 researchers, doctors, teacher-researchers, engineers, and healthcare personnel around patients, with the aim of accelerating research and diagnostic and therapeutic innovation to change the lives of families affected by genetic diseases. Labeled as an Institute Hospitalo-Universitaire (IHU) in 2011 and 2019 and a Carnot Institute in 2020, Institut *Imagine* is supported by its six founding members, including AP-HP, Inserm, and Université Paris Cité, as well as 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, with nearly half of them lacking a diagnosis and over 8 out of 10 having no dedicated treatment. Faced with this public health emergency, the challenge is twofold: to diagnose and to cure.

About Bpifrance

Bpifrance Investissement manages equity investments for Bpifrance, providing financing to companies at every stage of their development through loans, guarantees, and equity. Bpifrance supports innovation projects and international expansion, offering various products to facilitate export activities. In addition to financing, Bpifrance offers advisory services, networking, and acceleration programs for startups, SMEs, and intermediate-sized enterprises, with 50 regional branches ensuring effective support for entrepreneurs to meet their challenges.

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