



Genespire raises €46.6 million (~\$52 million) in a Series B round to advance its first pediatric *in-vivo* gene therapy into the clinic

One of the largest private Italian Biotech company financings to date

Financing co-led by Sofinnova Partners, XGEN Venture and CDP Venture Capital forming a strong syndicate with Indaco SGR

Funds dedicated to advancing off-the-shelf pediatric gene therapy for Methylmalonic Acidemia (MMA) into the clinic, and to bolstering the Company's gene therapy pipeline

Milan, ITALY – 25 September 2024 – Genespire, a biotechnology company developing off-the-shelf gene therapies for pediatric patients affected by genetic diseases, today announced the closing of a €46.6 million (~\$52 million) Series B financing, co-led by Sofinnova Partners, XGEN Venture and CDP Venture Capital through its Large Venture Fund forming a strong syndicate with Indaco SGR.

The financing will enable the development of GENE202, the company's lead candidate, up to a Phase I/II clinical trial for the treatment of MMA, a devastating genetic disorder impairing the metabolism of certain amino acids and fats.

MMA has onset in early infancy, is associated with significant mortality and morbidity, and is characterized by severe clinical manifestations including muscular weakness, seizures, developmental delays and organ damage. Currently, there are no disease-modifying treatments for MMA. GENE202 is a pioneering off-the-shelf gene therapy which harnesses the company's Immune Shielded Lentiviral Vector (ISLV) platform. ISLVs are designed to be used intravenously, enabling the patient's liver to produce the therapy throughout its lifetime. This unique mode of action makes Genespire's approach optimally suited to address pediatric patients affected by genetic diseases who currently face the most pressing unmet medical needs, while also benefitting adult patients. The ISLV technology was developed by Genespire's scientific co-founders and gene therapy leaders, Pr. Luigi Naldini and Dr. Alessio Cantore, at the San Raffaele Telethon Institute for Gene Therapy (SR-Tiget).

This Series B financing round will also strengthen the Company's pipeline by fostering discovery and preclinical work on candidate products targeting several additional genetic diseases.

Karen Aiach-Pignet, Genespire's CEO said, *"Our highly innovative ISLV platform gives Genespire the unique ability to bring groundbreaking treatments that have the potential to transform the lives of children suffering from genetic disease. The support from our new and existing investors reflects the enthusiasm and confidence in our platform's potential, with further validation achieved from the promising data generated to date. We extend a heartfelt gratitude to our previous CEO, Julia Berretta, for her instrumental role in this financing round, and we look forward to working closely with CDP Venture Capital, XGEN Venture and Indaco SGR, alongside our founding investor, Sofinnova Partners, as well as the SR-Tiget team, as we drive our first therapy in MMA towards the clinic."*

PRESS RELEASE

“Since our initial investment, we’ve been deeply convinced by Genespire’s world-class team and its groundbreaking lentiviral technology.” said Lucia Faccio, Partner at Sofinnova Partners. “We remain firmly committed to supporting the company’s growth in collaboration with our colleagues from XGEN, CDP Venture Capital and Indaco, to pave the way for single treatment approaches for several genetic disorders in children.”

“Gene therapy is starting to deliver results with several recent clinical successes and product approvals and we believe that Genespire’s approach offers huge opportunities. We look forward to working with fellow investors and the Management team to help realize this potential.” commented Paolo Fundarò, Founder and Managing Partner from XGEN Venture.

“Health, particularly Biotechnology Research dedicated to therapeutic needs, is one of the strategic macro-areas prioritized in our 2024-2028 Business Plan,” says Agostino Scornajenchi, CEO and General Manager of CDP Venture Capital, “Genespire’s research started in the renowned laboratories of the San Raffaele Institute, and is being advanced by an ambitious team that, through advanced gene therapy technologies and gene therapies, aims to offer curative treatments for diseases that currently lack effective therapeutic options, such as MMA. We look forward to supporting the company to set a new international gold standard.”

In connection with the Series B round, Marco Dieci, Senior Advisor at CDP Venture Capital and Chief Executive Officer of Simis S.r.l., will join Genespire’s Board of Directors as the representative for CDP Venture Capital, alongside Paolo Fundarò, Founder and Managing Partner from XGEN Venture.

Enquiries:

Genespire

Tel: +39 02 80896651
info@genespire.com

ICR Consilium

Amber Fennell/Ashley Tapp/Jonathan Edwards

Tel: +44 (0) 20 3709 5700
Genespire@consilium-comms.com

About Genespire

Genespire is a biotechnology company, developing off-the-shelf gene therapies based on immune shielded lentiviral vectors (ISLVs) for pediatric patients affected by genetic diseases. ISLVs are designed to be used intravenously and allow the life-long production of the therapy directly from the patient’s liver. Genespire is initially advancing therapeutic programs in inherited metabolic diseases with high unmet medical need. Based in Milan, Italy, Genespire was founded in March 2020 by the gene therapy pioneer Prof. Luigi Naldini and Dr. Alessio Cantore, at the Fondazione Telethon and Ospedale San Raffaele. Genespire is a spin-out of SR-Tiget, a world leading cell and gene therapy research institute. Find out more about us at www.genespire.com.

About Sofinnova Partners

Sofinnova Partners is a leading European venture capital firm in life sciences, specializing in healthcare and sustainability. Based in Paris, London and Milan, the firm brings together a team of professionals from all over the world with strong scientific, medical and business expertise. Sofinnova Partners is a hands-on company builder across the entire value chain of life sciences investments, from seed to later-stage. The firm actively partners with ambitious entrepreneurs as a lead or cornerstone investor to develop transformative innovations that have the potential to positively impact our collective future.

PRESS RELEASE

Founded in 1972, Sofinnova Partners is a deeply established venture capital firm in Europe, with 50 years of experience backing over 500 companies and creating market leaders around the globe. Today, Sofinnova Partners has over €2.8 billion under management. For more information, please visit: www.sofinnovapartners.com. For more information about the Sofinnova Telethon Strategy, visit: <https://sofinnovapartners.com/strategy/telethon>.

About XGEN Venture

XGEN Venture is a Milan-based, recently created life science venture capital firm. We work with a group of exceptional scientists and entrepreneurs to translate high level science into solutions for patients. Our company creation strategy identifies and supports the most convincing opportunities to build effective businesses and create value.

Investing in technologies that truly improve severe health conditions is at the core of what XGEN does and is committed to doing so in a responsible and impactful way, promoting best ESG practices within the meaning of SFDR art 8. For more information, please visit www.xgenventure.com.

About CDP Venture Capital

CDP Venture Capital is an asset management company participated by CDP Equity (70%) and Invitalia (30%) with the goal of making venture capital a cornerstone of Italy's economic development and innovation, creating the conditions for the overall, sustainable growth of the entire innovation ecosystem. CDP Venture Capital manages 13 investment funds, amounting to over 3 billion euros in resources that support innovative enterprises at all stages of their life cycle, making both direct and indirect investments (funds of funds).

For more information: www.cdpventurecapital.it

About Indaco Venture Partners SGR

Indaco Venture Partners SGR is one of the largest independent Italian venture capital asset management companies, with over €370 million in assets under management, focused on companies that innovate in electronics, robotics, new materials, medtech, biotech – pharma and digital. Indaco Bio Fund (around €100 million) is exclusively dedicated to investing in the development of innovative pharmacological therapies, across all therapeutic indications but with particular attention to Oncology, CNS, Ophthalmology, Metabolic diseases, Virology, and Infectious diseases. Indaco is focused on the Italian Biotech sector with select investments in other European countries, USA, Canada, and Israel.

About SR-Tiget

The San Raffaele Telethon Institute for Gene Therapy (SR-Tiget) was created in 1996 as a joint venture between the Fondazione Telethon and Ospedale San Raffaele, with the mission to perform cutting-edge research, towards the discovery of novel gene and cell therapy platforms and their development from preclinical models to first-in-human testing. For more information please visit [San Raffaele Telethon Institute for Gene Therapy - HSR Research](http://SanRaffaeleTelethonInstituteforGeneTherapy-HSRResearch)