

Genespire Secures €16 Million Series A Financing from Sofinnova Partners to Advance Transformative Gene Therapies

Biotech executive, Julia Berretta, Ph.D., is named Chief Executive Officer

MILAN, Italy — April 29, 2020 — Genespire, a biotechnology company focused on the development of transformative gene therapies for patients affected by genetic diseases, announced today the successful close of a €16M Series A financing from Sofinnova Partners, a leading European life sciences venture capital firm based in Paris, London and Milan. The company also announced the appointment of Julia Berretta, Ph.D., as Chief Executive Officer and member of the Board of Directors. Graziano Seghezzi, Managing Partner at Sofinnova Partners, and Lucia Faccio, Ph.D., Partner at Sofinnova Partners, will also join the Board.

Genespire was founded in March 2020 as a spin-off of the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget), one of the world's leading cell and gene therapy research institutes spearheaded by gene therapy pioneer Prof. Luigi Naldini. The Company was co-founded by Fondazione Telethon and the San Raffaele Hospital, along with Prof. Naldini and Dr. Alessio Cantore.

The funds will be used to advance Genespire's leading-edge platform technologies towards the development of novel gene therapies in two main areas: primary immunodeficiencies and metabolic genetic diseases.

"Our mission has always been to develop breakthrough solutions for genetic diseases," said Prof. Naldini, Genespire's co-founder and Director of SR-Tiget. "This financing enables the company to translate our innovative science and early stage programs into clinical development. The appointment of Dr. Berretta as CEO is a major reinforcement of our team."

Sofinnova Partners' Dr. Faccio added, "Genespire is an exciting investment with all the key ingredients for success: Outstanding scientists that developed the first ex-vivo gene therapy to market, experienced executives brought in through Sofinnova Partners' network and game changing technologies that have the potential to impact the lives of patients with genetic diseases."

"I am thrilled to be joining Genespire and such exceptional scientific founders," said Dr. Berretta. "Genespire was born of decades of experience in the gene therapy field, and is optimally positioned to advance transformative therapies for patients affected by severe inherited diseases."

Dr. Berretta was part of the Executive Committee of Cellectis S.A., a Nasdaq-listed clinical stage gene editing company developing CAR-T cell therapies for cancer, where she led business development as well as strategic planning. She is also an independent Board member of Treefrog Therapeutics, an innovative stem cell company.

About Genespire

Genespire is a biotechnology company focused on the development of transformative gene therapies for patients affected by genetic diseases, particularly primary immunodeficiencies and inherited metabolic diseases. Based in Milan, Italy, Genespire was founded in March 2020 by the gene therapy pioneer Prof. Luigi Naldini, Dr. Alessio Cantore, Fondazione Telethon and Ospedale San Raffaele. It is a spin-off of SR-Tiget, a world leading cell and gene therapy research institute and is backed by Sofinnova Partners. www.genespire.com

About Sofinnova Partners

Sofinnova Partners is a leading European venture capital firm specialized in Life Sciences. Based in Paris, France, with offices in London and Milan, the firm brings together a team of 40 professionals from all over Europe, the U.S. and Asia. The firm focuses on paradigm-shifting technologies alongside visionary entrepreneurs. Sofinnova Partners invests across the Life Sciences value chain as a lead or cornerstone investor, from very early-stage opportunities to late-stage/public companies. It has backed nearly 500 companies over more than 48 years, creating market leaders around the globe. Today, Sofinnova Partners has over €2 billion under management.

For more information, please visit: www.sofinnovapartners.com

About Fondazione Telethon

Fondazione Telethon is a non-profit organisation created in 1990 as a response to the appeals of a patient association group of stakeholders, who saw scientific research as the only real opportunity to effectively fight genetic diseases. Thanks to the funds raised through the television marathon, along with other initiatives and a network of partners and volunteers, Telethon finances the best scientific research on rare genetic diseases, evaluated and selected by independent internationally renowned experts, with the ultimate objective of making the treatments developed available to everyone who needs them. Throughout its 30 years of activity, Fondazione Telethon has invested more than € 528 million in funding more than 2.630 projects to study more than 570 diseases, involving over 1.600 scientists. Fondazione Telethon has made a significant contribution to the worldwide advancement of knowledge regarding rare genetic diseases and of academic research and drug development with a view to developing treatments. For more information, please visit: www.telethon.it

About Ospedale San Raffaele

Ospedale San Raffaele (OSR) is a clinical-research-university hospital established in 1971 to provide international-level specialised care for the most complex and difficult health conditions. OSR is part of *Gruppo San Donato*, the leading hospital group in Italy. The hospital is a multi-specialty center with over 60 clinical specialties; it is accredited by the Italian National Health System to provide care to both public and private, national and international patients. Research at OSR focuses on integrating basic, translational and clinical activities to provide the most advanced care to our patients. The institute is recognized as a global

authority in molecular medicine and gene therapy, and is at the forefront of research in many other fields. Ospedale San Raffaele is a first-class institute which treats many diseases and stands out for the deep interaction between clinical and scientific area. This makes the transfer of scientific results from the laboratories to the patient's bed easier. Its mission is to improve knowledge of diseases, identify new therapies and encourage young scientists and doctor to grow professionally. For more information, please visit: www.hsr.it

About the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget)

Based in Milan, Italy, the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) is a joint venture between the Ospedale San Raffaele and Fondazione Telethon. SR-Tiget was established in 1995 to perform research on gene transfer and cell transplantation and translate its results into clinical applications of gene and cell therapies for different genetic diseases.

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