



PRESS RELEASE

Genespire announces oral presentation at the ASGCT Annual Meeting detailing preclinical insights into dosing for first-in-human *in vivo* liver-directed ISLV gene therapy for MMA

Milan, ITALY – 8 May 2025 – Genespire, a biotechnology company developing off-the-shelf gene therapies for pediatric patients affected by genetic diseases, today announces an upcoming oral presentation showcasing critical preclinical insights into dosing for first-in-human *in vivo* liver-directed immune shielded lentiviral vector (ISLV) gene therapy for methylmalonic acidemia (MMA) at the American Society of Gene and Cell Therapy (ASGCT) 2025 Annual Meeting to be held May 13-17, 2025 in New Orleans.

Presentation details:

Oral presentation title: Definition of a Minimal Therapeutic Dose of *In Vivo* Liver-Directed Lentiviral Gene Therapy for Methylmalonic Acidemia

Session title: Gene and Cell Therapy for Metabolic Diseases

Date: Tuesday, May 13, 2025

Presenter: Dr. Elena Barbon, Research Scientist at the San Raffaele Telethon Institute for Gene Therapy (SR-Tiget) in collaboration with Genespire

Genespire is developing a novel proprietary class of lentiviral vectors, the Immune Shielded Lentiviral Vectors (ISLVs), with lead candidate GENE202 nearing clinical development for the treatment of methylmalonic acidemia (MMA), a devastating genetic disorder impairing the metabolism of certain amino acids and fats.

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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, 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.