

Unlocking Access: Cell & Gene Therapy at Scale

November 9-10, 2026 | Ospedale San Raffaele, Milan



Agenda Day 1

Monday, November 9

8:30 – 13:00	30 Years of SR-TIGET: Open to public
08:45	Welcome and Institutional Opening Remarks Chiara Bonini, OSR Director Ilaria Villa, Fondazione Telethon General Manager
09:00	30 Years of SR-TIGET Luigi Naldini, Director, SR-TIGET Maria Grazia Roncarolo, Former Director, SR-TIGET; Stanford University; Tr1X Inc
09:30	Wiskott–Aldrich Syndrome Gene Therapy: From Discovery to First-in-Human Trials and Approval Alessandro Aiuti, Deputy Director, SR-TIGET Jacob Thomas, Patient testimonial
10:15	Coffee break
10:45	Keynote Lecture: Genetic Engineering Hematopoiesis: From Gene Addition to Genome Editing Luigi Naldini, SR-TIGET, Italy
11:15	Keynote Lecture: Mutation-Specific, Mutation-Agnostic, and Disease-Agnostic Therapeutic Genome Editing David Liu, Broad Institute of MIT and Harvard, US
12:00	A Panel Discussion: Partnership as the Engine of Translation <i>Moderated by Jose-Carlos Gutierrez-Ramos, Danaher, US</i> Alessandro Aiuti, SR-TIGET, Milan Luca Alberici, AGC Biologics, Milan Sadik Kassim, Danaher, US Matthew Porteus, Stanford University, US Maria Grazia Roncarolo, Stanford University and Tr1X Inc, US Priya Stephen, WAS Foundation, US
13:00-14:30	Lunch break – Move to scientific session (registration required to attend)
14:30 – 17:30	HSC Gene Therapy: State of the Art, Achievements, and Remaining Challenges
14:30	How Hospital Innovation Can Reach Patients When There Is No Market: The Case of SCID-Artemis Inside the ATMP@AP-HP Platform Marina Cavazzana, Hôpital Necker-Enfants Malades, France
15:00	Toward Sustainable HSC Gene Therapy for Patients with Rare Diseases: Lessons from the Spanish Network on Advanced Therapies Juan A. Bueren, CIEMAT, Spain
15:30	25 Years of HSC Gene Therapy at GOSH Adrian J. Thrasher, Great Ormond Street Hospital, UK
16:00	Gene Editing in Thalassemia: From the Seminal Studies to the Real-World Application Franco Locatelli, Bambino Gesù Children's Hospital, Italy
16:30	Coffee break
17:00	HSC Gene Therapy in Lysosomal Storage Disorders: Therapeutic Benefit vs. Allo-HSCT, Long-Term Outcomes, and Ongoing Developments Maria Ester Bernardo, SR-TIGET, Italy
17:30 – 18:30	Safety and Clonal Dynamics in HSC Gene Therapy
17:30	Viral DNA Integration: Mechanisms and Consequences Frederic D. Bushman, University of Pennsylvania, US
18:00	Clonal Tracking in HSC Gene Therapy: Insights into Human Hematopoiesis and Long-Term Safety Eugenio Montini, SR-TIGET, Italy

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Agenda Day 2 Tuesday, November 10

08:45 – 09:15	Manufacturing Advanced Therapies
08:45	Designing Advanced Therapies That Survive Scale: From Early Decisions to Commercial Readiness Daria Donati, Cytiva, US
09:15 – 12:45	Novel Editing Technologies
09:15	The Next Generation of Genome Editing for Sickle Cell Disease: Toward Safer, More Accessible Curative Therapies John Tisdale, NIH, US
09:45	Generalist Editing Approaches for Bone Marrow Failure Daniel Bauer, Dana-Farber/Boston Children's Hospital, US
10:15	Asymmetric Collaborations to Unlock Genetically Engineered Stem Cells as Curative Drugs Matthew Porteus, Stanford University, US
10:45	Advancing Personalized Gene Editing Strategies for Fanconi Anemia Paula Rio, CIEMAT, Spain
11:15	Coffee Break
11:45	Engineering Protected Hematopoiesis to Unlock Safer and More Accessible Cell and Gene Therapies Pietro Genovese, Harvard Medical School, US
12:15	Targeting Neurometabolic and Neurodegenerative Diseases via Hematopoietic Stem Cell Gene Therapy Alessandra Biffi, Padua University Hospital, Italy
12:45	Closing Keynote: Advanced Therapies: From Breakthrough Science to Sustainable Access Claire Booth, Great Ormond Street Hospital, UK
13:15	Closing Remarks Tom Foti, Aldevron, US Luigi Naldini, SR-TIGET, Italy
13:30	Lunch & Departures