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** Chairs: Fabio Ciceri and Chiara Bonini
08:45	**Welcome and Institutional Opening Remarks** Chiara Bonini, OSR Scientific 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 Chair: Chiara Bonini
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 Chair: Fabio Ciceri
10:15	**Coffee break**
10:45	**Keynote Lecture: Genetic Engineering Hematopoiesis: From Gene Addition to Genome Editing** Luigi Naldini, SR-TIGET, Italy Chair: Fabio Ciceri
11:15	**Keynote Lecture: Mutation-Specific, Mutation-Agnostic, and Disease-Agnostic Therapeutic Genome Editing** David Liu, Broad Institute of MIT and Harvard, US Chair: Chiara Bonini
12:00	**A Panel Discussion: Partnership as the Engine of Translation** *Moderated by Jose-Carlos Gutierrez-Ramos, Danaher, US* Alessandro Aiuti, SR-TIGET, Italy Luca Alberici, AGC Biologics, Italy Jennifer Moody, 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** Chairs: Fabio Ciceri and Daria Donati
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** Chairs: Fabio Ciceri and Daria Donati
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** Chairs: Giuliana Ferrari and Aaron Dulgar-Tulloch
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** Chairs: Giuliana Ferrari and Aaron Dulgar-Tulloch
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 Chair: Tom Foti
13:15	**Closing Remarks** Tom Foti, Aldevron, US Luigi Naldini, SR-TIGET, Italy
13:30	**Lunch & Departures**

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