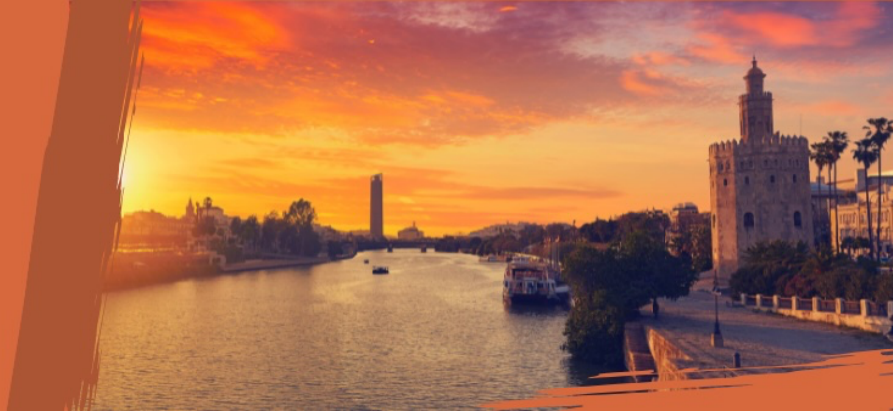
Homologous Directed Repair
1-Day Workshop

Seville, Spain
6 October 2025



Monday, 6 October, 2025 (10:00 am – 6:30 pm)

Opening Remarks/Introduction **Matthew Porteus, MD, PhD**	
10:00 – 10:05am	
Session 1 **Chairs – Rasmus Bak, PhD/Ayal Hendel, PhD**	
10:05 – 10:25am	***"Modulation of DNA repair to improve homology dependent recombination and primed repair"*** Marcello Maresca, PhD Senior Director, Genome Engineering - BioPharma R&D, AstraZeneca
10:25 – 10:45am	***"Enhancing CRISPR-Cas Genome Editing in Human Hematopoietic Stem and Progenitor Cells Using HDR Enhancers"*** Ayal Hendel, PhD Associate Professor, Bar-Ilan University
10:45 – 11:05am	***"Circularization of Single-Stranded DNA Donor Template Unleashes the Power of Non-Viral Gene Delivery for HSPC editing"*** Julien Valton, PhD Vice President Gene Therapy, Cellectis. SA
11:05 – 11:20am	***"From Editing to Engineering - A New Role for Base Editors: Repurposing base editors for targeted knock-in and simultaneous knock-outs to generate multiplex-edited allogeneic CAR T cells with minimal translocations"*** Viktor Glaser Berlin Center for Advanced Therapies Charité Universitätsmedizin Berlin
11:20 – 11:35am	***"Integration Frequency of Full and Truncated AAV Genomes in Cas9-Induced DSB Sites Is Determined by DNA Repair Pathways"*** Gloria Gonzalez-Aseguinolaza CIMA Universidad de Navarra
Break (25-minutes)	

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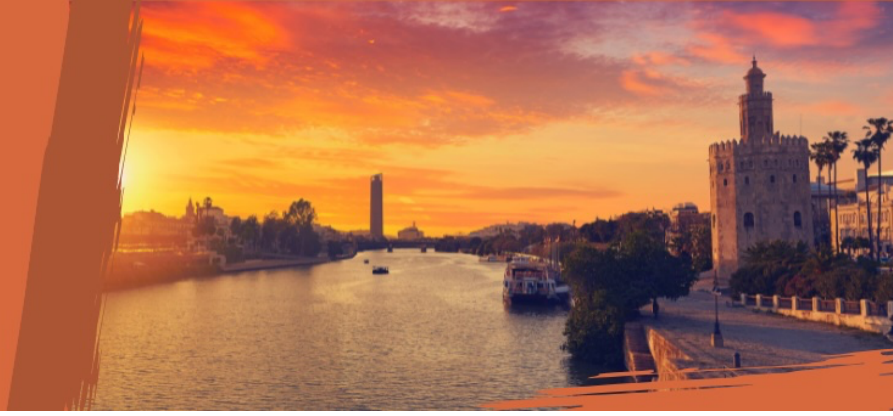

Session 2
Chairs – Alessia Cavazza, PhD/Matt Porteus, MD, PhD

12:00 – 12:20pm	***"Mechanistic insights to further advance gene and cell therapy applications"*** Raffaella Di Micco, PhD Group Leader at SR-TIGET, Milan Associate Professor at IUSS, Pavia
12:20 – 12:40pm	***"Characterization of hematopoietic stem cell fitness and safety for the clinical application of a scalable HDR-based CRISPR/Cas9 gene editing platform"*** Alessia Cavazza, PhD Associate Professor in Gene Therapy Zayed Centre for Research into Rare Diseases in Children
12:40 – 1:00pm	***"Advancing Therapies for Inborn Errors of Immunity Through Gene Editing"*** Rasmus Bak, PhD Associate Professor Aarhus University
1:00 – 1:15pm	***"Novel homology-mediated end joining -IDLV precise integration for Therapeutic Genome Editing in Hematopoietic Stem Cells"*** Giulia Scalisi, PhD Postdoctoral Scholar, Therapeutic genome editing team, Genethon, Inserm Integrare research unit UMR_S951
1:15 – 1:30pm	***"Optimized Gene Editing in Human HSPCs Enhances Efficiency While Preserving Polyclonal Engraftment"*** Isabel Ojeda Perez CIEMAT

Lunch (60-minutes)

Session 3
Chairs – Justin Eyquem, PhD/David Rawlings, MD

2:30 – 2:50pm	***"In vivo HDR to reprogram T cells"*** Justin Eyquem, PhD Associate Professor Division of Hematology/Oncology University of California San Francisco

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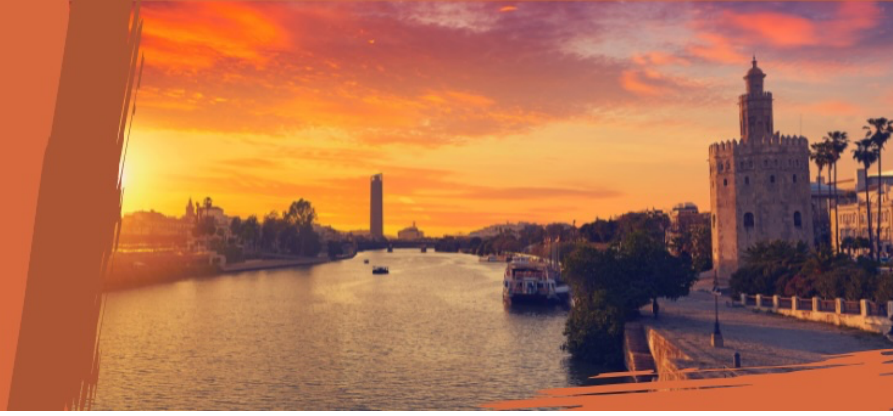

2:50 – 3:10pm	***"Modeling Therapeutic Application of HDR Engineered Regulatory T cells"*** David Rawlings, MD Director, Center for Immunity and Immunotherapies Seattle Children's Research Institute Professor of Pediatrics and Immunology, University of Washington School of Medicine
3:10 – 3:30pm	***"Leveraging homology-directed repair for engineering B cell-based immunotherapies"*** Richard James, PhD Associate Professor, Pediatrics and Pharmacology Seattle Children's Research Institute
3:30 – 3:45pm	***"One-pot PASTA: Enhanced targeted integration of large transgenes in human T cells via HDR-guided landing pad insertion and serine recombinases"*** Isabell Kassing Charité Universitätsmedizin Berlin
3:45 – 4:00pm	***"Enabling gene therapy with Megabulb DNA -- a novel circular single-stranded CRISPR editing template"*** Elena Stoyanova, PhD Touchlight DNA Services

Break (30-minutes)

Session 4
Chairs – Matt Porteus MD, PhD/Jose Carlos Segovia Sanz, PhD

4:30 – 4:50pm	***"Translating Homology Directed Repair into the Clinic"*** Matt Porteus, MD, PhD Professor of Pediatrics Member Institute of Stem Cell Biology and Regenerative Medicine Director, Center for Definitive and Curative Medicine Sutardja Chuk Professor of Definitive and Curative Medicine Stanford University
4:50 – 5:10pm	***"HDR based Gene Editing Therapy for Pyruvate Kinase Deficiency"*** Jose Carlos Segovia Sanz, PhD Head of the Cell Technology Division CIEMAT-CIBERER / IIS-FJD

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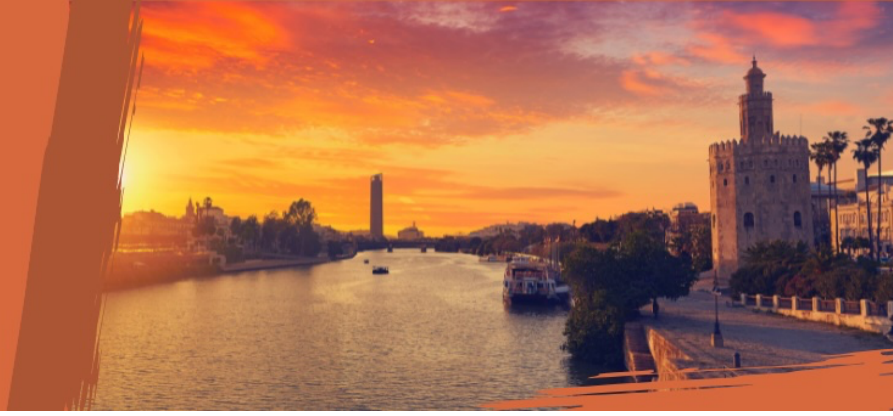

5:10 – 5:30pm	***"Translating gene-size editing strategies of hematopoietic cells to the treatment of inherited diseases"*** Luigi Naldini, MD, PhD Director, San Raffaele Telethon Institute for Gene Therapy (SR-Tiget) Professor of Tissue Biology and Gene Therapy Vita - Salute San Raffaele University Medical School
5:30 – 5:45pm	***"Targeted gene insertion for functional CFTR restoration in Cystic Fibrosis airway epithelium"*** Brian R. Davis, Ph.D.; Professor and Director Gregory Fleming James Cystic Fibrosis Research Center Nancy R. and Eugene C. Gwaltney Family Endowed Chair in Medical Research Department of Medicine; Division of Pulmonary, Allergy, and Critical Care Medicine Heersink School of Medicine; The University of Alabama at Birmingham

Cocktail Hour/Networking

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