Gene Therapy in Fanconi Anemia: Placing and Replacing FA Genes

Thursday, July 8, 2021

Link to Register: https://cvent.me/ZbeGXm

TIME (CET)	TALK TITLE & SPEAKER
16:00 pm - 16:05 pm	Meeting Welcome *Isis Sroka, Fanconi Anemia Research Fund, USA*
16:05 pm - 16:10 pm	Chair Introductions *Co-chairs: Paula Río & Juan Bueren, CIEMAT/CIBERER/Fundación Jiménez Díaz, Spain*
SESSION 1| LENTIVIRAL-MEDIATED GENE THERAPY	
16:10 pm	Keynote Introduction *Juan Bueren, CIEMAT/CIBERER/Fundación Jiménez Díaz, Spain*
16:10 pm - 16:40 pm	**KEYNOTE ADDRESS: Lentiviral and Targeted Gene Therapy of Hematopoietic Stem Cells for the Treatment of Inherited Diseases** **Luigi Naldini, San Raffaele Telethon Institute for Gene Therapy, Italy**
16:40 pm - 16:55 pm	Lessons learned 5 years after starting the FANCOLEN-1 Gene Therapy Trial *Juan Bueren, CIEMAT/CIBERER/Fundación Jiménez Díaz, Spain*
16:55 pm - 17: 05 pm	Molecular and Clinical Impact of Eltrombopag in FA patients treated by Gene Therapy *Julián Sevilla, The Hospital Infantil Universitario Niño Jesús, Spain*
17:05 pm - 17: 15 pm	Gene Therapy Restores the Transcriptional Program of Fanconi Anemia Hematopoietic Stem Cells *David Gómez, Navarrabiomed, Complejo Hospitalario de Navarra (CHN), Universidad Pública de Navarra (UPNA), IdiSNA, Spain*
17:15 pm - 17:30 pm	**Group Discussion**
17:30 pm - 17:40 pm	Gene Therapy for Fanconi Anemia [Group A]: Interim Results from RP-L102 Global Clinical Trials *Agnieszka Czechowicz, Stanford University, USA*
17:40 pm - 17:50 pm	Gene Therapy for Fanconi Anemia: Progress in the Global RP-L102 Phase II Trials and Future Perspectives *Jonathan Schwartz, Rocket Pharmaceuticals, USA*
17:50 pm - 18: 10 pm	**Group Discussion**
18:10 pm - 18:25 pm	Break
SESSION 2 | GENE EDITING	
18:25 pm	Welcome Back Introduction *Paula Río, CIEMAT/CIBERER/Fundación Jiménez Díaz, Spain*
18: 25 pm - 18:40 pm	Reversing FA genotypes in patient cells with CRISPR-Cas base and prime editing *Jacob Corn, ETH Zurich, Switzerland*

18:40 pm - 18:55 pm	Digital Correction of Fanconi Anemia Patient Mutations for Gene Therapy *Branden Moriarity, University of Minnesota, USA*
18:55 pm - 19:10 pm	Correction of Fanconi Anemia Mutations by Homology Directed Repair-Independent Gene Editing Strategies *Paula Río, CIEMAT/CIBERER/Fundación Jiménez Díaz, Spain*
19:10 pm - 19:40 pm	**Group Discussion**
19:40 pm - 20:00 pm	Application of Crispr-Cas9 Gene Editing for the Treatment of Genetic Disorders *Sandeep Soni, University of California, San Francisco and Crispr Therapeutics, Inc., USA*
20:00 pm - 20:30 pm	**KEYNOTE ADDRESS: Ex Vivo and In Vivo Base Editing to Rescue Genetic Diseases in Animals** **David Liu, Harvard University, USA**
20:30 pm - 20:50 pm	**Final Group Discussion**
20:50 pm	FARF Meeting Close *Isis Sroka, Fanconi Anemia Research Fund, USA*
20:52 pm	Spatial.Chat Virtual Meetups

* There are no poster abstracts for the Gene Therapy Day.