International Society
ISCT
Cell & Gene Therapy®

Novel Ex-Vivo *Gene Therapy* Approaches and Emerging Gene Editing Technologies

September 12, 2019
MADISON, WI, USA

ISCT SCIENTIFIC *Signature* SERIES

ISCT *Signature* SERIES

Novel Ex-Vivo *Gene Therapy* Approaches and Emerging Gene Editing Technologies

Thursday, September 12, 2019 • 8:30 – 16:15
Monona Terrace Community and Convention Center, Madison, USA

Sandeep Soni, MD, Stanford University, Palo Alto, CA, United States

Developed by the ISCT Immuno-Gene Therapy Committee, this full day symposium offers the unique opportunity to gather key opinion leaders in the field of Gene Therapy to discuss, present and develop position statements to move the field forward.

Aimed at driving thought leadership by addressing key issues and building collaboration, the ISCT Gene Therapy Signature Series will address a variety of novel ex-vivo gene therapy approaches and emerging gene editing technologies from the translational science, manufacturing and regulatory perspectives.

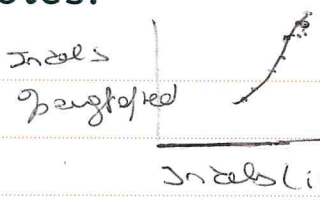
Program

SESSION 1 – HSPC SELECTION, CONDITIONING AND THE SYNTHETIC BIOLOGY PLATFORM	
08:30 – 10:00	**TARGETING A HIGHLY ENRICHED HEMATOPOIETIC STEM CELL POPULATION FOR GENE THERAPY AND EDITING** *Hans-Peter Kiem, MD, PhD, Fred Hutchinson Cancer Research Center, USA*
	A CD117-AMANITIN ANTIBODY DRUG CONJUGATE (ADC) EFFECTIVELY DEPLETES HUMAN AND NON-HUMAN PRIMATE HEMATOPOIETIC STEM AND PROGENITOR CELLS (HSPCS): TARGETED NON-GENOTOXIC CONDITIONING FOR BONE MARROW TRANSPLANT *Anthony Boitano, PhD, Magenta Therapeutics, USA*
	MANUFACTURING DEVELOPMENT OF ALLOGENEIC CELL THERAPIES POWERED BY GENE CIRCUITS *Philip Lee, PhD, Senti Biosciences, USA*
	OPEN DISCUSSION (30 MIN)
10:00 – 10:15	Coffee Break
SESSION 2 – THE CRISPR-CAS GENE EDITING SYSTEM	
10:15 – 11:45	**PROGRESS AND CHALLENGES IN DEVELOPING A CAS9 BASED HEMATOPOIETIC STEM CELL DRUG** *Matthew Porteus, MD, PhD, Stanford University, USA*
	NHEJ-MEDIATED GENE EDITING: AN ALTERNATIVE APPROACH TO EFFICIENTLY CORRECT HSPCS FROM PATIENTS WITH FANCONI ANEMIA *Paula Rio, PhD, Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), SPA*
	ROLE OF P53 IN CRISPR-CAS9 EDITING *Robert Ihry, PhD, Novartis Institute of Biomedical Research, USA*
	OPEN DISCUSSION (30 MIN)

Program

11:45 – 12:45	Lunch
SESSION 3 – ADVANCES IN GENE THERAPY TECHNOLOGIES AND APPLICATIONS	
12:45 – 14:30	**PRECISE GENE EDITING OF MUTANT ALLELES USING CRISPR-CAS9 RIBONUCLEOPROTEIN COMPLEXES** *Krishanu Saha, PhD, University of Wisconsin-Madison, USA*
	GENE EDITING FOR FETAL HEMOGLOBIN INDUCTION *Daniel Bauer, MD, PhD, Boston Children's Hospital and Harvard Medical School, USA*
	USE OF LENTIVIRAL VECTORS IN HSCS FOR TREATMENT OF MONOGENIC DISEASE *Melissa Bonner, PhD, bluebird bio, USA*
	ESTABLISHING CGMP MANUFACTURING OF CRISPR/CAS9-EDITED HUMAN CAR T CELLS *Isabelle Rivière, PhD, Memorial Sloan Kettering Cancer Center, USA*
	OPEN DISCUSSION (30 MIN)
14:30 – 14:45	Coffee Break
SESSION 4 – REGULATORY & MANUFACTURING CONSIDERATIONS	
14:45 – 16:15	**NUANCES OF GENE THERAPY PRODUCT MANUFACTURING, ANALYSIS AND QUALITY TESTING** *Sarah Nikiforow, MD, PhD, Dana-Farber Cancer Institute, USA*
	DEVELOPING A CGMP MANUFACTURING PROCESS FOR CELL AND GENE THERAPIES *Neehar Bhatia, PhD, Stanford University, USA*
	ADVANCING INDIVIDUALIZED GENE THERAPY *Peter Marks, MD, PhD, Center for Biologics Evaluation and Research, FDA, USA*
	OPEN DISCUSSION (30 MIN)

Notes:



Different tools to test off target for clinical applicat

Buscar una nuclease FDA para trabar

Human genetic variation can modify specif and off target activity

UDiTAS™ → genome editing detection method of indels ...

& Revisar microhomology