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Boston Children's Hospital
Gene Therapy Program



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**13th Stem Cell Clonality and
Genome Stability Retreat**
October 23rd, 2023
Brussels, Belgium



Sponsored by the *Transatlantic Gene Therapy Consortium*

Organizing Committee: Christopher Baum, Claire Booth, Pietro Genovese, Don Kohn, Caroline Kuo, Sung-Yun Pai, Axel Schambach, Adrian Thrasher, Anna Villa, and David Williams

Monday, October 23rd, 2023

7:30 am - 8:30pm: Breakfast & On-Site Registration

Welcome/Opening Remarks

8:00 am David Williams on behalf of Organizing Committee

Editing I

Chair: Pietro Genovese

8:05 – 8:25 am **Angelo Lombardo:** Development and Application of Epigenetic Editing Strategies for Gene and Cell Therapy

8:25 – 8:45 am **Mike Holmes:** Writing DNA with RNA: Correcting the Sickle Cell Disease Mutation in Patient HSC Using RNA Gene Writers, an All-RNA Based Approach to Edit the Genome by Target Primed Reverse Transcription

8:45 – 9:05 am **Dan Bauer:** Approaches to Monitor and Mitigate Genotoxicity of HSC Gene Editing Recognizing Human Genetic Diversity

9:05 – 9:25 am **Jacob Stewart-Ornstein:** Prime Editing in Hematopoietic Diseases and Beyond: Efficacy and Safety

9:25 – 9:35 am Discussion/ Q&A

9:35 – 9:55 am COFFEE BREAK

Editing II

Chair: Don Kohn

9:55 – 10:15 am **Annarita Miccio:** Editing Strategies for Beta-Hemoglobinopathies

10:15 – 10:35 am **Paula Rio:** Expanding the Application of Precise Genome Editing to Fanconi Anemia

10:35 – 10:55 am **Samuel Sternberg:** Development of CRISPR-Associated Transposases for DSB-Free Targeted DNA integration

10:55 – 11:05 am Discussion/ Q&A

Keynote

11:05 – 11:50 am **Luigi Naldini:** Advancing Genetic Engineering of Hematopoiesis to Improve Precision and Broaden Its Therapeutic Applications

11:50 – 12:00 pm Discussion/ Q&A

12:00 – 1:00 pm LUNCH

In Vivo Delivery I

Chair: Axel Schambach

1:00 – 1:20 pm **Christian Brendel:** Engineering Retroviral Vectors for *In Vivo* HSC Gene Therapy

1:20 – 1:40 pm **Luca Biasco:** *In Vivo* Delivery of Genetic Payloads to Human Hematopoietic Stem/Progenitor Cells

1:40 – 2:00 pm **Stefano Rivella:** RNA Delivery by LNP Technology

2:00 - 2:10 pm Discussion/ Q&A

In Vivo Delivery II

Chair: Christopher Baum

2:10 – 2:30 pm **Jennifer Hamilton:** Cell Type-Programmable CRISPR-Cas9 Delivery for Human T Cell Engineering *In Vivo*

2:30 – 2:50 pm **Els Verhoeyen:** Nanoblades Allow High-Level Genome Editing in Murine and Human Organoids

2:50 – 3:10 pm **David Scadden:** Therapeutic Potential of *In Vivo* Cryogel-Based Bone Marrow Organoids

3:10 – 3:20 pm Discussion/ Q&A

3:20 – 3:40 pm COFFEE BREAK

Cellular Immunological Responses

Chair: Sung-Yun Pai

3:40 – 4:00 pm **Anna Kajaste-Rudnitski:** Dissecting Innate Immunity and Nucleic Acid Sensing of Viral Vectors

4:00 – 4:08 pm (8) **Don Kohn:** Loss of Engraftment of Gene Corrected HSC in XCGD

4:08 - 4:28 pm **Marieke Essers:** Interferon Signaling Heterogeneity at the Level of the Stem Cell

4:28 – 4:38 pm Discussion/ Q&A

Clinically Relevant Vector Insertion

Chair: Claire Booth

4:38 – 4:58 pm **Eugenio Montini:** Adaptive Routes of Hematopoietic Stem Cell Differentiation to Disease Conditions & Age in Gene Therapy Patients

4:58 – 5:06 pm (8) **Harry Malech:** Mechanism, Effect and Correction of a Cryptic Splicing Element in the Insulator of an X-SCID Lentivirus Vector

5:06 – 5:14 pm (8) **Daniela Cesana:** First Case Of Leukemia In Retroviral Gene Therapy For ADA-SCID

5:14 – 5:24 pm Discussion/ Q&A

Late Breaking Talks

Chair: Adrian Thrasher

5:25 – 5:45 pm **Hans-Peter Kiem:** Targeting Approaches for HSC Gene Therapy/Editing

5:45 – 6:05 pm **Speaker:** Title

6:05 – 6:15 pm Discussion/ Q&A

Closing Remarks/Announcements

6:15 – 6:20 pm Axel Schambach on Behalf of Organizing Committee

6:30 pm Post-Retreat Networking Session