



From 8:45

Welcome

9:20-9:30

Introduction (Sébastien Maury/Gaëlle Sandillon)

Session 1: Casgevy, where do we stand?

9:30-9:55

Gonzalo de Luna & Laure Joseph: Patients treated with Casgevy in France: where are we?

9:55-10:20

Anne Hulin: Individualized busulfan dosing prior to gene therapy

10:20-10:55

Nicolas Hébert: Hemolysis markers and cellular distribution of hemoglobins

20mn break

11:15-11:55

INVITED SPEAKER - Mohsen Alzahrani (Riyadh, Saudi Arabia): Gene Therapy for Sickle Cell Disease and β -Thalassemia: Real-World Experience, Challenges, and Future Directions

Session 2: Managing the risks (1/2)

11:55-12:20

Thomas d'Humières: Managing cardiovascular risks before and after gene therapy or hematopoietic stem cell transplantation

12:20-12:55

Ivan Sloma: Monitoring cellular clonality before and after gene therapy or hematopoietic stem cell transplantation

12:55-14:10 Lunch break

Session 2: Managing the risks (2/2)

14:10-14:35

Mario Amendola: Monitoring off-target modifications following gene therapy

14:35-15:00

Julian Boutin: High-resolution, in-depth assessment of large chromosomal rearrangements at the cleavage site following CRISPR gene therapy for red blood cell disorders

15:00-15:25

Anne Galy: Building safer cell and gene therapies

20mn break

Session 3: Therapeutic innovations and future perspectives

15:45-16:10

Sandrine Visentin and Sarah Szeptowski: Current standard care for thalassemia major. Role of including allogeneic transplantation.

16:10-16:35

Bérengère Koehl: Prospects for treating hemoglobinopathies in pediatrics

16:35-16:45

Closing remarks

AP-HP