

iFeTIS 2025
UCSF Mission Bay Campus
Genentech Hall, Byers Auditorium
600 16th Street, San Francisco, California
Sunday, October 19, 2025

8:30 **Continental Breakfast** (pick-up name badge)

9:10 **Welcome – Tippi MacKenzie, MD, UCSF**

PRENATAL DIAGNOSIS

9:20-9:40 Genome Sequencing to Inform in Utero Therapy: Update on Prenatal
Diagnosis
Mary Norton, MD, UCSF
Q&A

9:40-10:00 Diagnosis and Treatment of Hydrops Fetalis
Teresa Sparks, MD, MAS, UCSF
Q&A

10:00-10:20 Prenatal Exome Sequencing 'Actionability' in the Era of Prenatal Gene
Therapy: Patient Perspectives
Julia Brown, PhD, UCSF
Q&A

10:20-10:35 **DISCUSSION**: Aligning Diagnostic and Therapeutic Arms of Precision
Medicine
Moderator: Dr. Lyn Chitty, University College London
(15 min)

10:35-11:00 **Break** (25 min)

CENTRAL NERVOUS SYSTEM

11:00-11:20 Accelerating Genetic Medicine Development
Stephan Sanders, BMBS, PhD, University of Oxford
Q&A

11:20-11:40 Technical Issues Related to Fetal Therapy Delivery for CNS Diseases
Nalin Gupta, MD, PhD, UCSF
Q&A

11:40-12:00 In Vivo Delivery of mRNA with Non-immunogenic Lipid Nanoparticles
Niren Murthy, PhD, UCB/IGI
Q&A

12:00-12:20 Engineering Stem Cells, Extracellular Vesicles, and Gene Editing for In Utero Treatment of Birth Defects
Aijun Wang, PhD, UCD
Q&A

12:20-12:40 Neurovascular Development and the Blood-Brain Barrier
Elizabeth Crouch, MD, PhD, UCSF
Q&A

12:45-1:50 **LUNCH** (65 min)

HEMATOLOGY

1:50-2:10 Prenatal Transplantation of a Clinical Cell Product Engineered to Secrete FVIII in a Sheep with Hemophilia A Corrects the Severe Bleeding Phenotype and Prevents Hemarthroses
Graça Almeida-Porada, MD, PhD, Wake Forest School of Medicine
Q&A

2:10-2:30 Enhancing Donor Cell Engraftment in Prenatal Hematopoietic Stem Cell Transplantation with Novel Antibody-Drug Conjugates
Christopher Porada, PhD, Wake Forest School of Medicine
Q&A

2:30-2:50 Update on In Utero HSCT for Fanconi Anemia
Agnieszka Czechowicz, MD, PhD, Stanford School of Medicine
Q&A

2:50-3:10 Lentiviral Vectors for HSC Gene Therapy for Alpha Thalassemia
Eva Segura, PhD, UCLA
Q&A

3:10-3:30 Virus-Like Particle-mediated In Utero Gene Therapy for Krabbe Disease
Stavros Loukogeorgakis, MBBS, PhD, University College London
Q&A

3:30-3:45 DISCUSSION:
(15 min)

3:45-4:05 **Break** 20 min

4:05-4:35 **Poster Session** 30 min

4:35-4:55 Funding Opportunities for In Utero Therapies from NHLBI
Lisbeth Welniak, PhD, NHLBI
Q&A

4:55-5:15 Opportunities and Bottlenecks for Gene Therapy and Gene Editing

Mimi Lee, MD, PhD, ARPA-H

Q&A

5:15-5:40

The Germline and the Limits of Medicine: Reflections on the Ethical
Horizons of Fetal Gene Therapy

Ben Hurlbut, PhD, Arizona State University

Q&A

5:40

Adjourn

6:00

Dinner – SF Kebab Mediterranean Grill

UCSF Mission Bay Campus

550 16th Street

SPONSORS



iFeTIS 2025
UCSF Mission Bay Campus
Genentech Hall, Byers Auditorium
Monday, October 20, 2025

8:30 **Continental Breakfast**

8:55 **Welcome Back – Tippi MacKenzie**

GENOME EDITING

9:00-9:20 Genome Editing for Hemoglobinopathies
M. Kyle Cromer, PhD, UCSF
Q&A

9:20-9:40 In Vivo HSC Editing
Atesh Worthington, PhD, UCSF
Q&A

9:40-10:00 Fetal Gene Editing for Cystic Fibrosis
David Stitelman, MD, Yale School of Medicine
Q&A

10:05-10:30 **Break** 25 min

GENOME EDITING (continued)

10:30-10:50 Prenatal Therapy for Sickle Cell Disease: Advancing Curative Strategies
Before Birth
Panicos Shangaris, MBBCh, PhD, King's College London
Q&A

10:50-11:10 IPEX Syndrome Diagnosis and Treatment
Rosa Bacchetta, MD, Stanford School of Medicine
Q&A

11:10-11:25 **DISCUSSION:**
(15 min)

11:25-11:45 Funding Opportunities for In Utero Therapies from NINDS
Chris Boshoff, PhD, NINDS
Q&A

11:45-12:50 **LUNCH** 65 min

GETTING TO THE CLINIC

- 12:50-1:10 Non-human Primate Models
Matthew Lawrence, PhD, Virscio
Q&A
- 1:10-1:30 Lessons from Umbilical Vein Injections
Juan M. González, MD, MS, PhD, UCSF
Q&A
- 1:30-1:50 Clinical Trial of Prenatal Enzyme Replacement Therapy for Lysosomal
Storage Disorders
Akos Herzeg, MD, PhD, MBA
Q&A
- 1:50-2:10 A Points-to-Consider Framework for Responsible Innovation
Sebastien Mazzuri, MD, MBA
Q&A
- 2:15-2:40 **Break** 25 min

GETTING TO THE CLINIC (continued)

- 2:40-3:00 Prenatal Sirolimus for STRADA-associated Polyhydramnios,
Megalencephaly, and Symptomatic Epilepsy Syndrome (PMSE)
Whitney Parker, MD, PhD, University of Maryland School of Medicine
Q&A
- 3:00-3:20 Prenatal mTOR inhibitors for Tuberous Sclerosis Complex
Jennifer L. Cohen, Duke University School of Medicine
Q&A
- 3:20-3:40 Prenatal AAV9/GLB1 Gene Therapy for GM1
Tippi C. MacKenzie, MD, UCSF
Q&A
- 3:45-4:30 **Round Table: Opportunities and Bottlenecks for Prenatal Therapies**
Moderators: Graça Almeida-Porada and Tippi MacKenzie
- 4:30 **Adjourn**

SPONSORS

