

**International Fetal Transplantation and Immunology Society
&
Wake Forest Institute for Regenerative Medicine**

Present



6th iFeTIS Meeting 2020

***From the Bench to the Bedside: Safely Translating Scientific
Knowledge to Clinical Applications***

This two-day virtual meeting arranged by iFeTIS and virtually hosted by the Fetal Research and Therapy Center, Wake Forest Institute for Regenerative Medicine, will cover the very latest research and clinical progress in the areas of prenatal diagnosis, cell and gene therapy, transplantation, fetal imaging and intervention, tissue engineering, regenerative medicine, risk stratification for in utero therapies, and ethics.

Scientific Committee

Graça Almeida-Porada
Tippi MacKenzie
Christopher Porada

Organizing Committee

Graça Almeida-Porada
Emily Gregg
Christopher Porada
Joan Schanck

<https://www.fetaltherapies.org>

6th iFeTIS Meeting 2020

Virtual

Agenda (EST Time Zone)

Thursday, October 1st, 2020

8:30 **Stage Room Open**

8:50-9:00 **Welcome**
Chris Porada, PhD, President iFeTIS

Session 1: Prenatal Therapies

9:00-9:30 **Regenerative Medicine: Current Concepts and Changing Trends**
Anthony Atala, MD

9:30-10:00 **Fetal Cell and Gene Therapy: A Perspective**
Chris Porada, PhD

10:00-10:30 **Development of Novel Treatments for In-Utero Use: FDA Perspective**
Larissa Lapteva, MD

10:30-11:00 **Advances in Noninvasive Prenatal Genetic Testing**
Mary Norton, MD

11:00-11:30 **Round Table: *Prenatal Therapies***
Chairpersons: Drs. Tippi Mackenzie, Paul Tam, Lisbeth Welniak
(All morning speakers)

11:30-12:00 **Gene Therapy Strategies for Cystic Fibrosis Lung Disease**
Paul B. McCray, Jr., MD

12:00-12:30 **Advancing Drug Design Principles for Gene Therapy**
Chris Doering, PhD

12:30-12:50 **Prenatal Therapy for Hemophilia A**
Graça Almeida-Porada, MD, PhD

12:50-1:00 **Questions and Answers**

01:00-1:20 **Break (20 minutes)**

Session 2: In Utero Gene Therapy Using AAV Vectors

- 1:20-1:50 **AAV Capsid Engineering with the Goal of Achieving Cell-Specific Targeting *in vivo***
Aravind Asokan, PhD
- 1:50:2:10 **Rationale for in Utero Therapy for Spinal Muscular Atrophy**
Charlotte Jane Sumner, MD
- 2:10-2:30 **In Utero Gene Therapy for SMA Mice**
Afrooz Rashnonejad, PhD
- 2:30-2:50 **In Utero CRISPR-Mediated Gene Editing in Small and Large Animals**
William Peranteau, MD
- 2:50-3:20 **Round Table: *In Utero Gene Therapy Using AAV vectors***
Chairpersons: Drs. Agnieszka Chechowicz and Trent Spencer

Session 3: Ongoing Clinical Trials and New Centers

- 3:20-3:40 **Osteogenesis Imperfecta**
Cecilia Götherström, PhD
- 3:40-4:00 **Establishing a Fetal Research Initiative at an Existing Fetal Clinical Center**
Amy O'Connell, MD, PhD
- 4:00-4:20 **Alpha Thalassemia Clinical Trial**
Tippi C. MacKenzie, MD
- 4:20-4:50 **Funding Trends and Opportunities at NHLBI and NIH for Prenatal Cell and Gene Therapy**
Lisbeth Welniak, PhD
- 4:50-5:20 **Round Table: *Clinical Trials and New Centers***
Chairpersons: Drs. Anna David and Magnus Westgren
- 5:20-5:30 **Happy Hour**
- 5:30 **Stage Room Closes**

Friday, October 2nd, 2020

8:45-9:00 **Stage Room Open**

Session 4: In Utero HSC Transplantation

9:00-9:20 In Utero Priming of the Fetal Immune System
Archita Mishra, PhD

9:20-9:40 In Utero Stem Cell Transplantation for the Haemoglobinopathies: Challenges and Limitations
Citra Mattar, MD

9:40-10:00 Optimal donors for IUHSCT: Options and Limitations
Karin Blakemore, MD

10:00-10:20 Mapping Human Hematopoietic Stem Cell Development
Hanna K.A. Mikkola, MD, PhD

10:20-10:40 **Round Table: *In Utero HSC Transplantation***
Chairpersons: Drs. Allistair Abraham, Graça Almeida-Porada, Paul Tam

Session 5: Gene Therapy for Hematological Disorders & Prenatal Treatment for Lysosomal Storage Disorders

10:40-11:10 Gene Therapy Applications for Sickle Cell Disease
John Tisdale, MD

11:10-11:30 Particle-Based Non-Nuclease Fetal Editing for Hemoglobinopathy
David Stitelman, MD

11:30-11:50 In utero gene Therapy for Fanconi Anemia
Agnieszka Czechowicz MD, PhD

11:50-12:10 Early Diagnosis and Treatment of Infantile-Onset Pompe Disease via Newborn Screen: Where We Are Now and How We Can Move Forward
Jennifer L. Cohen, MD

12:10-12:30 Enzyme Replacement Therapy for Lysosomal Storage Disorders
Tippi C. MacKenzie, MD

12:30-1:00 **Round Table: *Gene Therapy for Hematological Disorders & Prenatal Treatment for Lysosomal Storage Disorders***
Chairpersons: Drs Lisbeth Welniak, Simon Waddington, Allistair Abraham

1:00-1:30 Break

Session 6: Prenatal Treatments of CNS Pathologies

- 1:30-1:50 Might the in Utero Administration of Neural Stem Cells Play a Role in the Amelioration of Neurological Diseases & Injuries?
Evan Y. Snyder, MD, PhD
- 1:50-2:10 Perinatal Gene Therapy for Dopamine Transporter Deficiency Syndrome
Joanne Ng, MBChB, MRCPCH, PhD
- 2:10-2:30 Fetal Intervention for Vein of Galen Malformations
Darren Orbach MD, PhD
- 2:30-2:50 Prenatal Tissue Engineering: Surgical Considerations
Paolo de Coppi, MD

Submitted Abstract:

- 2:50-3:00 Preclinical Stem Cell Therapy in Fetuses with Myelomeningocele: A Systematic Review and Meta-analysis
Yada Kunpalin, MD
- 3:00-3:30 **Round Table: *Prenatal Treatments of CNS Pathologies***
Chairpersons: Drs. P. Ellen Grant, and Simon Waddington

Session 7: Fetal Intervention, Risks and Ethics

- 3:30-3:50 Fetal Intervention
Brian Brost, MD
- 3:50-4:10 The Role of MRI in Fetal Therapies
P. Ellen Grant, MD
- 4:10-4:30 Risk Stratification for in Utero Therapies
Anna David, PhD
- 4:30-4:50 Ethical Clinical Trials in Utero: Progress, Precautions, and Prospects
Nancy King, JD
- 4:50-5:20 Round Table: *Fetal Intervention, Risks and Ethics***
Chairpersons: Drs. Paolo de Coppi, Darren Orbach
- 5:20-5:50 ***Final Round Table: Moving Forward***
Chairpersons: Drs. Anna David, Chris Porada

***Meet the Participants
(Alphabetical Order)***

Allistair Abraham, MD
Attending Physician, Division of Blood and Marrow Transplantation
Children's National Hospital
Assistant Professor of Pediatrics
George Washington University School of Medicine and Health Sciences



Graça Almeida-Porada, MD, PhD
Professor of Regenerative Medicine
Director, Fetal Research and Therapy Program
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Aravind Asokan, PhD
Director of Gene Therapy
Professor in Surgery Professor of Molecular Genetics and Microbiology
Duke University School of Medicine



Anthony Atala, MD
W.H. Boyce Professor of Urology
G. Link Professor
Director of Wake Forest Institute for Regenerative Medicine
Chair, Department of Urology
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Karin Blakemore, MD
Director, Prenatal Genetics, Prenatal Diagnostic Center
Professor of Gynecology and Obstetrics
Johns Hopkins Medicine
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Brian Brost, MD
Section Head of Maternal-Fetal Medicine
Wake Forest Baptist Health
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Jerry Chan Kok Yen, MD, PhD
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Jennifer L. Cohen, MD
Pediatric Medical Genetics Specialist
Assistant Professor of Pediatrics
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Agnieszka Czechowicz, MD, PhD
Assistant Professor of Pediatrics
Pediatric Stem Cell Transplantation
Stanford University



Anna David, PhD
Professor of Obstetrics and Maternal Fetal Medicine
Director, EGA Institute for Women's Health,
University College London (UCL)



Paolo de Coppi, MD
NIHR Professor
Nuffield Professor of Paediatric Surgery
Great Ormond Street Hospital for Children
Honorary Professor, Wake Forest University
Honorary Professor, University of Padova



Christopher Doering, PhD
Professor of Pediatrics - Cell and Gene Therapy
Aflac Cancer & Blood Disorders Center
Emory University School of Medicine



Cecilia Götherström, PhD
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Department of Clinical Science, Intervention and Technology – CLINTEC
Karolinska Institute and Karolinska University Hospital



P. Ellen Grant, MD
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Center
Professor of Radiology and Pediatrics
Harvard Medical School
Boston Children's Hospital



Nancy King, JD
Professor, Social Sciences and Health Policy
Professor, Wake Forest Institute for Regenerative Medicine
Co-Director, Center for Bioethics, Health, & Society
and Graduate Program in Bioethics,
Maya Angelou Center for Health Equity
Wake Forest School of Medicine



Larissa Lapteva, MD
Associate Director
Division of Clinical Evaluation, Pharmacology, and Toxicology
Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research (CBER)
U.S. Food and Drug Administration (FDA)



Tippi C. MacKenzie, MD
Professor of Surgery
Division of Pediatric Surgery
Co-Director Fetal Treatment Center
University of California, San Francisco



Paul B. McCray, Jr., MD
Professor of Pediatrics - Pulmonary Medicine
Professor of Microbiology and Immunology
Associate Director of the Center for Gene Therapy
University of Iowa



Citra Mattar, MD
Assistant Professor, Department of Obstetrics & Gynaecology
Consultant, Department of Obstetrics & Gynaecology
National University Hospital
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Hanna K.A. Mikkola, MD, PhD
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Broad Stem Cell Research Institute
University of California, Los Angeles



Mary Norton, MD
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Vice Chair, Clinical and Translational Genetics
Director, Program in Genetic and Genomic Prenatal Medicine
UCSF Benioff Children's Hospital
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Amy O'Connell, MD, PhD
Physician in Medicine
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Darren Orbach MD, PhD
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Sage Schermerhorn Chair in Image-Guided Therapy
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Christopher Porada, PhD
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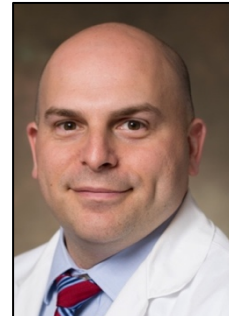
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