Wake Forest School of Medicine

Institute for Regenerative Medicine

**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

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| 8:30 | **Stage Room Open** |
| 8:50-9:00 | **Welcome**<br>Chris Porada, PhD, President iFeTIS |

**Session 1: Prenatal Therapies**

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| 9:00-9:30 | **Regenerative Medicine: Current Concepts and Changing Trends**<br>Anthony Atala, MD |
| 9:30-10:00 | **Fetal Cell and Gene Therapy: A Perspective**<br>Chris Porada, PhD |
| 10:00-10:30 | **Development of Novel Treatments for In-Utero Use: FDA Perspective**<br>Larissa Lapteva, MD |
| 10:30-11:00 | **Advances in Noninvasive Prenatal Genetic Testing**<br>Mary Norton, MD |
| 11:00-11:30 | **Round Table**: ***Prenatal Therapies***<br>Chairpersons: Drs. Tippi Mackenzie, Paul Tam, Lisbeth Welniak<br>(All morning speakers) |
| 11:30-12:00 | **Gene Therapy Strategies for Cystic Fibrosis Lung Disease**<br>Paul B. McCray, Jr., MD |
| 12:00-12:30 | **Advancing Drug Design Principles for Gene Therapy**<br>Chris Doering, PhD |
| 12:30-12:50 | **Prenatal Therapy for Hemophilia A**<br>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
Jerry Chan Kok Yen, MD, 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 Hematopoietic Ontogeny and Fetal Bone Marrow Niche
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
Simon Waddington, 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 Magnus Westgren

## 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
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Assistant Professor of Pediatrics
George Washington University School of Medicine and Health Sciences

Graça Almeida-Porada, MD, PhD
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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
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G. Link Professor
Director of Wake Forest Institute for Regenerative Medicine
Chair, Department of Urology
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Karin Blakemore, MD
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Professor of Gynecology and Obstetrics
Johns Hopkins Medicine
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Brian Brost, MD
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Jerry Chan Kok Yen, MD, PhD
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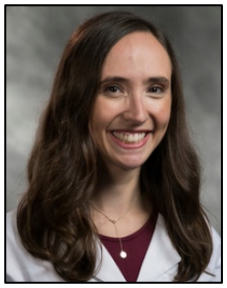

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Nuffield Professor of Paediatric Surgery
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Honorary Professor, Wake Forest University
Honorary Professor, University of Padova

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