

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

*Present*



***8<sup>th</sup> iFeTIS Meeting 2024 on the 10<sup>th</sup> anniversary of iFeTIS***

***November 1<sup>st</sup> and 2<sup>nd</sup>***

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

This two-day in-person meeting arranged by iFeTIS and 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, regenerative medicine, cell and gene therapy, transplantation, fetal imaging and intervention, tissue engineering, regulatory pathways and ethics.

**Scientific Committee**

Graça Almeida-Porada, M.D., Ph.D.  
Anthony Atala, M.D.  
Karin Blakemore, M.D.  
Tippi MacKenzie, M.D.  
Christopher Porada, Ph.D.

**Organizing Committee**

Graça Almeida-Porada, M.D., Ph.D.  
Christopher Porada, Ph.D.  
Anthony Atala, M.D.  
  
Karri Adams, EdD.  
Laurie Bonilla  
Joan Schanck, M.P.A.

# Agenda

## (EST Time Zone)

### Friday, November 1<sup>st</sup>, 2024

- 7:15-8:00      Registration Open**
- 7:15-8:00      Continental Breakfast**
- 8:00-8:10      Welcome 10<sup>th</sup> Anniversary of iFeTIS**  
Graça Almeida-Porada, MD, PhD, President iFeTIS
- 8:10-8:20      Welcome from WFIRM**  
Anthony Atala, MD, Director of the Wake Forest Institute for Regenerative Medicine

#### Session1: Fetal Intervention, Diagnosis, Approaches, Risks, and Clinical Trials

- 8:20-8:40      Regenerative Medicine: Current Concepts and Applications to Fetal Therapies**  
Anthony Atala, MD
- 8:40-9:00      Advances in Noninvasive Prenatal Genetic Testing using Whole Exome Sequencing**  
Mary Norton, MD (Video talk)
- 9:00-9:20      Fetal Intervention for Vein of Galen Malformations**  
Darren Orbach MD, PhD
- 9:20-9:40      Update on the CuRe Trial for Myelomeningocele**  
Diana Farmer, MD, FACS

- 9:40-10:00      Round Table: *The Advantages and Risks of Fetal Interventions***  
All session speakers  
Chairpersons: Drs. Stephen Sawyer, Paolo de Coppi

**10:00-10:10      Coffee Break**

#### Session 2: Ethics and Regulatory Aspects of Prenatal Therapies

- 10:10-10:30      Ethics of Fetal Therapies**  
Alta Charro, JD
- 10:30-10:50      Patients Attitudes Towards Prenatal Therapy**  
Julia Brown, PhD and Emma Canepa, MS,CCRP
- 10:50-11:10      Use of MFAET: Maternal Fetal Adverse Event Terminology and proposed new version 2.0 terms**  
Rebecca Spencer, MD, PhD

**11:10-11:30 Scientists' Role in Science Advocacy**

Zubin Master, PhD

**11:30-11:50 Development of Novel Treatments for In-Utero Use: FDA Perspective**

Najat Bouchkouj, MD (Video Talk)

**11:50-12:10 Regulatory Perspectives on Fetal Therapies**

Steven Bauer, PhD

**12:10- 1:00 Round Table: Safety, Regulatory, and Ethics of Prenatal Therapies**

All session speakers

**Chairpersons:** Drs. Graça Almeida-Porada, Tippi MacKenzie

**1:00-2:00 LUNCH and**

**1:15-1:45 Roundtable Discussion on Manufacturing of Age Bespoke Products**

Drs. Sébastien Mazzuri, Stephen Sawyer

**Session 3: In Utero and Postnatal Stem Cell Transplantation**

**2:00-2:20 Development of the Fetal Immune System**

Trevor Burt, MD

**2:20-2:40 Fetal Immune System in Mid-Gestation**

Jerry Chan Kok Yen, MD, PhD

**2:40-3:00 Creating and Validating a Sheep Model of Sickle Cell Disease for Testing the Safety and Efficacy of Novel Prenatal Treatments**

Christopher Porada, PhD

**3:00-3:20 A Phase I Clinical Trial for Hemophilia A Using Lentiviral Transduced Hematopoietic Stem Cells with a Myeloid-Directed and Enhanced-Expression Factor VIII Transgene**

H. Trent Spencer, PhD

**3:20-3:40 Prenatal Therapy for Hemophilia A**

Graça Almeida-Porada, MD, PhD

**3:40-4:00 NHLBI Priorities and Funding Mechanisms for Research into Fetal and Neonatal Interventions**

Lisbeth Welniak, PhD

**4:00-4:20 Round Table: In Utero and Postnatal Stem Cell Transplantation**

All session speakers

**Chairpersons:** Drs. Karin Blakemore, Bill Peranteau

**4:20-4:30 Coffee Break**

**4:30-4:40 Short talks selected from abstracts:**  
**ES-derived MSC for Hypophosphatasia**  
Naoya Kitamura

**Session 4: Novel Models to Predict Efficacy and Safety**

**4:40-5:00 Combining Natural and Artificial Intelligence in Gene Therapy Candidate Design**  
Chris Doering, PhD

**5:00-5:20 Amniotic Fluid Organoids (AFO) for Diagnosis and Therapy**  
Paolo de Coppi, MD, PhD

**5:20-5:40 Utilizing Amniotic Fluid Cells for Fetal Arrhythmia Diagnosis and Therapeutic Testing**  
Joshua Maxwell, PhD

**5:40-6:00 Lung Organoids to Test Drug delivery**  
Sean Murphy, PhD

**6:00-6:30 Round Table: Novel Models to Predict Efficacy and Safety**  
**All session speakers**

**Chairpersons:** Drs. Steven Bauer, Stephen Sawyer, Chris Porada,

**6:30 -7:30- Speakers' Dinner**

## Saturday, November 2<sup>nd</sup>, 2024

**7:30-8:00 Registration Open**

**7:30-8:00 Continental Breakfast**

### **Session 5: From the Bench to the Clinic: Neuromuscular Disorders**

**8:00-8:20 Screening for Lipid Nanoparticles for Duchenne Muscular Dystrophy Genome Editing**

Baisong Lu, PhD

**8:20-8:40 Prenatal Intra-Amniotic Antisense Oligonucleotide Therapy for Spinal Muscular Atrophy**

Beltran Borges, MD

**8:40-9:00 Prenatal Therapy for SMA with Risdiplam – A Case Report**

Richard Finkel, MD

**9:00-9:20 Experience with Postnatal AAV9 Gene Therapy for Children with GM1.**

Cynthia J. Tifft, MD, PhD

**9:20-9:40 Overview of Relevant NINDS Funding Opportunities**

Chris H. Boshoff, PhD

**9:40-10:00 Round Table: Neuromuscular Disorders**

**All session speakers**

**Chairpersons:** Drs Tippi Mackenzie, Diana Farmer

**10:00-10:10 Coffee Break**

### **Session 6: Nanoparticle Delivery for In Utero Genome Editing**

**10:10-10:30 Engineering Extracellular Vesicles and Lipid Nanoparticles for in utero Treatment of Structural and Genetic Birth Defects**

Aijun Wang, PhD, FAIMBE

**10:30-10:50 Particle-Based Epigenetic Therapy to Improve Lung Development**

David Stitelman, MD

**10:50-11:10 Targeted Gene Editing of HSC in Utero**

William Peranteau, MD

**11:10-11:30 Prenatal Therapy for Sickle Cell Disease**

Panicos Shangaris, AFHEA, MRCOG, PhD

**11:30-11:50 Prenatal Therapy for Fanconi Anemia**

Agnieszka Czechowicz, MD, PhD

**11:50-12:10 Round Table: Gene Delivery**

**All session speakers**

**Chairpersons:** Drs. Trent Spencer, Chris Doering

**12:10-1:00 LUNCH and**

**12:20-12:50 Round Table on Equity, Education, and Workforce Development**

Drs. Brenda A. Latham-Sadler, Russ Read

**Session 7: From the Bench to the Clinic: Lysosomal Storage Disorders**

**1:00-1:20 Enzyme Replacement Therapy for Lysosomal Storage Disorders- Updates from the PEARL Trial**

Tippi C. MacKenzie, MD

**1:20-1:40 Advancing Precision care in Pregnancy Through a Treatable Fetal Findings List**

Jennifer L. Cohen, MD

**1:40- 2:10 Round Table: Treating Lysosomal Storage Disorders in Utero**

**All session speakers**

**Chairpersons:** Drs. Karin Blakemore and Sébastien Mazzuri

**Session 8: Clinical Trial for XLHED**

**2:10-2:30 Prenatal Therapy for XLHED**

Agnès Jaulent, PhD

**2:30- 3:40 Questions and Answers**

**3:40-4:30 Round Table: The Future of Prenatal Therapies**

All speakers invited to participate

**Chairpersons:** Drs. Graça Almeida-Porada, Tippi MacKenzie

**4:30 Adjourn**

**4:30 Tour Institute for Regenerative Medicine and Test Bed**

## Meet the Speakers (Alphabetical Order)

### **Anthony Atala, MD**

W.H. Boyce Professor of Urology

G. Link Professor

Director of Wake Forest Institute for Regenerative Medicine

Chair, Department of Urology

Wake Forest School of Medicine



### **Graça Almeida-Porada, MD, PhD**

Professor of Regenerative Medicine

Director, Fetal Research and Therapy Program

Wake Forest Institute for Regenerative Medicine

Wake Forest School of Medicine



### **Steven Bauer, PhD**

Chief Regulatory Science Affairs

Wake Forest Institute for Regenerative Medicine



### **Karin Blakemore, MD**

Director, Prenatal Genetics, Prenatal Diagnostic Center

Professor of Gynecology and Obstetrics

Johns Hopkins Medicine

The John Hopkins University



**Najat Bouchkouj, MD**

Medical Officer  
Malignant Hematology Branch  
Division of Clinical Evaluation Hematology  
Office of Clinical Evaluation  
Office of Therapeutic Products  
FDA/CBER



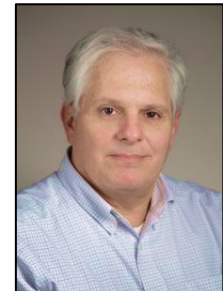
**Beltran Borges, MD**

Department of Surgery, UCSF  
UCSF Center for Maternal-Fetal Precision Medicine  
The Eli and Edythe Broad Center of Regeneration Medicine  
and Stem Cell Research at UCSF



**Chris H. Boshoff, PhD**

Program Director  
Division of Translational Research  
National Institute of Neurological Disorders & Stroke  
National Institutes of Health



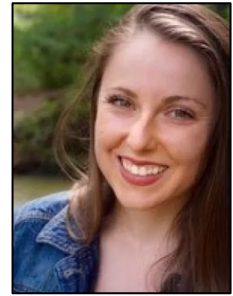
**Trevor Burt, MD**

Associate Professor of Pediatrics  
Neonatology, Pediatrics  
Duke University School of Medicine





**Emma Canepa MS, CCRP**  
Program Manager  
University of California, San Francisco



**Jerry Chan MD, PhD**  
Professor, Experimental Fetal Medicine Group  
KK Women's and Children's Hospital  
Duke National University of Singapore



**Alta Charo, JD**  
Warren P. Knowles Professor of Law and Bioethics Emeritus  
University of Wisconsin–Madison



**Jennifer L. Cohen, MD**  
Pediatric Medical Genetics Specialist  
Assistant Professor of Pediatrics  
Division of Medical Genetics  
Duke University



**Agnieszka Czechowicz, MD, PhD**  
Assistant Professor of Pediatrics  
Pediatric Stem Cell Transplantation  
Stanford University



**Paolo de Coppi, MD, PhD**  
NIHR Professor  
Nuffield Professor of Paediatric Surgery  
Great Ormond Street Hospital for Children



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



**Diana L. Farmer, MD, FACS, FRCS**  
Chairperson, Department of Surgery  
Surgeon-in-Chief, UC Davis Children's Hospital  
Distinguished Professor and Pearl Stamps Stewart Endowed Chair  
Founder and Co-Director, Department of Surgery Center for Surgical  
Bioengineering



**Richard S. Finkel, MD**

Director, Center for Experimental Neurotherapeutics  
George J. Pedersen Endowed Chair in Neurotherapeutics  
St. Jude Children's Research Hospital



**Dr. Brenda Latham-Sadler**

Vice-Chief Academic Officer,  
Justice, Equity, Diversity & Inclusion (JEDI)  
Clinical Professor Family and Community Medicine  
Affiliate in the Maya Angelou Center for Health Equity



**Baisong Lu, PhD**

Associate Professor  
Fetal Research and Therapy Program  
Wake Forest Institute for Regenerative Medicine  
Wake Forest School of Medicine



**Agnès Jaulent, PhD**

Project Leader and Alliance Manager  
EspeRare Foundation - Advancing treatments for rare diseases together  
Geneva, Switzerland



**Tippi C. MacKenzie, MD**  
Professor of Surgery  
Division of Pediatric Surgery  
Co-Director Fetal Treatment Center  
Director of the UCSF Broad Stem Cell Center  
University of California, San Francisco



**Zubin Master, PhD**  
Associate Professor  
Social Sciences and Health Policy  
Wake Forest School of Medicine



**Joshua T. Maxwell, PhD**  
Assistant Professor  
Wake Forest Institute for Regenerative Medicine  
Wake Forest School of Medicine



**Sébastien Mazzuri, MD**  
Prenatal Platform Director EspeRare



**Sean Vincent Murphy, PhD**

Associate Professor  
Wake Forest Institute for Regenerative Medicine  
Wake Forest School of Medicine



**Mary Norton, MD**

Professor, Ob/Gyn, Reproductive Sciences  
Vice Chair, Clinical and Translational Genetics  
Director, Program in Genetic and Genomic Prenatal Medicine  
UCSF Benioff Children's Hospital  
University of California, San Francisco



**Darren Orbach MD, PhD**

Chief, Neurointerventional Radiology  
Co-Director, Cerebrovascular Surgery and Interventions Center  
Sage Schermerhorn Chair in Image-Guided Therapy  
Associate Professor of Radiology  
Boston Children's Hospital  
Harvard Medical School



**William H. Peranteau, MD**

Attending Surgeon, Division of General, Thoracic, and Fetal Surgery  
Children's Hospital of Philadelphia  
Assistant Professor of Surgery, Perelman School of Medicine  
University of Pennsylvania



**Christopher Porada, PhD**

Professor of Regenerative Medicine  
Fetal Research and Therapy Program  
Wake Forest Institute for Regenerative Medicine  
Professor School of Biomedical Engineering and Sciences  
Wake Forest School of Medicine



**Russ Read, GradDipEd**

Executive Director for the National Center for the Biotechnology Workforce  
Program  
Forsyth Tech Community College



**Stephen W Sawyer, PhD**

Assistant Professor of Regenerative Medicine  
Wake Forest Institute for Regenerative Medicine  
Professor School of Biomedical Engineering and Sciences  
Wake Forest School of Medicine



**Panicos Shangaris MSc, AFHEA, MRCOG, PhD**

Senior Clinical Lecturer and Consultant in Maternal & Fetal Medicine  
Department of Women and Children's Health, & Peter Gorer Department of  
Immunobiology King's College London  
Senior Clinical Lecturer and Consultant in Maternal & Fetal Medicine  
Department of Women and Children's Health, & Peter Gorer Department of  
Immunobiology King's College London



**H. Trent Spencer, PhD**

Professor, Department of Pediatrics  
Emory University School of Medicine  
Director, Gene and Cell Therapy  
Aflac Cancer and Blood Disorders Center  
Georgia Cancer Coalition Distinguished Scholar



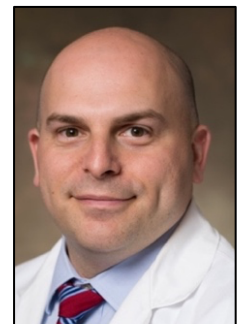
**Rebecca Spencer, MD, PhD**

NIHR Clinical Lecturer and Subspecialty Trainee in Maternal and Fetal  
Medicine  
University of Leeds  
UK



**David Stitelman, MD**

Assistant Professor of Surgery (Pediatrics)  
Assistant Professor of Surgery (Pediatrics) and Obstetrics, Gynecology &  
Reproductive Sciences  
Division of Pediatric Surgery  
Yale University School of Medicine





**Cynthia J. Tift, MD, PhD**

Deputy Clinical Director & Senior Clinician  
National Human Genome Research Institute  
Director, Pediatric Undiagnosed Diseases Program  
National Institutes of Health



**Aijun Wang, PhD, FAIMBE**

Chancellor's Fellow Professor of Surgery and Biomedical Engineering  
Co-Director, Center for Surgical Bioengineering  
Vice Chair for Translational Research, Innovation and  
Entrepreneurship | Department of Surgery | UC Davis School of Medicine  
Principal Investigator, Institute of Pediatric Regenerative Medicine (IPRM),  
Shriners Children's, Northern California



**Lisbeth Welniak, PhD**

Program Director  
Division of Blood Diseases and Resources  
National Heart, Lung, and Blood Institute

