

## Pediatric Clinical Trials Workshop for Early Career Investigators

**Date/Time:** Friday, May 1, 2020, 8:00 AM – 4:30 PM  
**Location:** Pediatric Academic Societies (PAS) 2020, Philadelphia, PA, Convention Center  
**PAS Event Type:** Pre-Conference Ancillary Event  
**Target Audience:** 50-100 early career investigators, their mentors, and senior research team members

### Learning Objectives:

- Compare and contrast traditional and innovative trial designs and how they are applied to advance pediatric therapeutic and device development.
- Describe the steps and requirements in the lifecycle of a pediatric clinical trial (including unique regulatory and ethical requirements).
- Explain how academic investigators, sponsors, and oversight entities collaborate in medical product development.
- Identify practical ways to minimize compliance and operational issues while managing a pediatric clinical trial.

**Abstract:** Although pediatric clinical trials present unique scientific and operational challenges, few training programs exist to prepare investigators to conduct complex pediatric research protocols. The Institute for Advanced Clinical Trials (I-ACT) for Children, Tufts Clinical and Translational Science Institute (CTSI), and Children's Hospital of Philadelphia (CHOP) with support from the Food and Drug Administration (FDA) will host a workshop to help early stage investigators conduct investigator-initiated, federally-funded, or industry-sponsored trials (especially multisite). This case-based, interactive day will feature collaborative instruction from government, industry, and academic experts. Keynote speakers will address the unique aspects of enrolling children in clinical trials and novel approaches to accelerate new product development. Industry leaders will discuss why it is difficult to develop drugs and devices for infants and children. The program will feature modules on study concept, design, start-up, study management, and close-out. Each module will include expert commentaries, panel discussions, and review of national and global resources available to support clinical trials. The workshop will emphasize the essential elements of quality management and broadly-engaged team science.

**Pre-work:** Participants will be expected to complete pre-work introductory material identified by the workshop organizers and speakers to effectively participate in case-based discussion.

### Tentative Detailed Program Agenda:

**07:30-08:00** Registration/Breakfast (30 min)

**08:00-08:15** **OPENING** (15 min)

**Welcome/Introduction/Resources** (15 min)

Moderator: Jonathan Davis (Tufts CTSI)

Jonathan Davis (Tufts), Gary Noel (I-ACT for Children), Lorraine Katz (CHOP)

**08:15-08:45** **KEYNOTE ADDRESS I** (30 min)

Moderator: Jonathan Davis (Tufts CTSI)

**Susan McCune** (FDA) *Unique aspects of enrolling children in clinical trials and novel approaches to accelerate new product development* (20 min)

Q&A (10 min)

**08:45-10:15** **MODULE 1: STUDY CONCEPTS AND DESIGN** (90 min)

Moderator: Gary Noel (I-ACT for Children)

**Module Introduction/Table Discussion** (20 min)

Gary Noel (I-ACT for Children)

*Introduction to Case Scenarios (5 min) and Table Discussion (15 min)*

**Panel Lightning Talks: Responses to the Case Scenarios** (55 min)

- Ron Portman (Novartis) *Innovative Trial Design Methods (alternative to RCT)* (10 min)
- Norma Terrin (Tufts CTSI) *Sample Size/Co-Enrollment* (10 min)
- Donna Snyder (FDA) *Ethics* (10 min)
- Diva De Leon (CHOP) *Investigator-Initiated Trials* (10 min)
- Lorraine Katz (CHOP) *NIH Consortium Trials* (10 min)

**Panel Discussion/Module Wrap-Up** (15 min)

Collin Hovinga (I-ACT for Children) *Responses to the Case Scenarios*

**10:15-10:35** **Break** (20 min)

**10:35-12:05** **MODULE 2: STUDY START-UP AND APPROVAL** (90 min)

Moderator: Jonathan Davis (Tufts CTSI)

**Module Introduction/Table Discussion** (20 min)

- Gerri Baer (FDA) *Data Needed to Support Initiating a Pediatric Study* (10 min)
- Collin Hovinga (I-ACT for Children) *Table Discussion on Cases* (10 min)

**Panel Lightning Talks: Responses to the Case Scenarios** (55 min)

- Kelly Wade (CHOP) *Protocol Feasibility* (10 min)
- Lisa Benson (I-ACT for Children) *Budgets/Contract Approval* (10 min)
- Mary Short (Lilly) *Study Metrics* (10 min)
- Brenda Poindexter (Cincinnati) *IND/IDE; How do I know I need one?* (10 min)
- Barbara Engel (CHOP) *IRB Approval (Role of IRB, Reliance Model, sIRB)* (10 min)

**Panel Discussion/Module Wrap-Up** (15 min)

Jonathan Davis (Tufts CTSI)/Collin Hovinga (I-ACT for Children) *Responses to the Case Scenarios*

**12:05-12:35** **Break/Boxed Lunch** (30 min)

**12:35-01:15** **PARTICIPANT INTRODUCTIONS AND KEYNOTE ADDRESS II** (40 min)

Moderator: Lorraine Katz (CHOP)

Participant Introductions (10 min)

**Thomas Miller** (Bayer) Why is it so difficult to develop new drugs or devices for infants and children? (20 min)

Q&A (10 min)

**01:15-02:45** **MODULE 3: STUDY CONDUCT AND MANAGEMENT** (90 min)

Moderator: Gary Noel (I-ACT for Children)

**Module Introduction/Table Discussion** (20 min)

Lisa Benson (I-ACT for Children) ,

*Case Scenarios (5 min) and Table Discussion (15 min)*

**Panel Lightning Talks: Responses to the Case Scenarios** (55 min)

- Charles Thompson (Pfizer) *Patient Engagement/Recruitment/Retention/Digital Methods* (10 min)
- Brian Smith(Duke) *Informed Consent/Assent/Dissent* (10 min)
- Lynne Yao (FDA) *Data Quality/Protection/Confidentiality* (10 min)
- Michael Blum (FDA) *Safety Reporting* (10 min)
- Lisa Guay-Woodford (Children's National) *CTSA Resources for Clinical Trials* (10 min)

**Panel Discussion/Module Wrap-Up** (15 min)

Lisa Benson (I-ACT for Children) *Responses to the Case Scenarios*

**02:45-03:05** **Break** (20 min)

**03:05-04:00** **MODULE 4: STUDY CLOSE-OUT** (55 min)

Moderator: Lisa Guay-Woodford (Children's National)

**Module Introduction/Table Discussion** (10 min)

Collin Hovinga (I-ACT for Children)

*Case Scenarios (5 min) and Table Discussion (5 min)*

**Panel Lightning Talks: Responses to the Case Scenarios** (35 min)

- Alexandra Mangili (Takeda) *Data Analysis* (10 min)
- Steven Hirschfeld (USU) *Publication* (10 min)
- Alberto Ortiz-Osorno (I-ACT) *Results Dissemination* (10 min)

**Panel Discussion/Module Wrap-Up** (10 min)

Jonathan Davis/Collin Hovinga (I-ACT for Children) *Responses to the Case Scenarios*

**04:00-04:30** **NEXT STEPS AND ASSESSMENT/EVALUATION** (30 min)

**Closing Remarks and Next Steps**

Jonathan Davis (Tufts), Gary Noel (I-ACT for Children), Lorraine Katz (CHOP) (15 min)

**Workshop Evaluation** (15 min)