

ADEPT 7: Advancing the Development of Pediatric Therapeutics Complex Innovative Trial Design

**FDA/UMD PUBLIC WORKSHOP
1-2 September 2021 (Virtual)**

Workshop Objective:

- **Provide a platform for scientific exchange among the FDA, other global health authorities, patient advocates, and drug developers**
 - **Discuss application and challenges of each topic in pediatric drug development and explore how challenges in the designs can be overcome**
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Day 1: September 1st 10am-1:15pm

Opening remarks:

10:00-10:10 am: Welcome/Introduction (John Alexander, FDA)

10:10-10:25 am: Setting the stage (Lynne Yao, FDA)

Session 1: Bridging biomarkers in Pediatric Extrapolation

Co-chairs: Lily Mulugeta (FDA) /Christine Garnett (FDA)

10:25-11:05 am: Regulatory perspective

- **10:25-10:35: FDA Perspective: Pulmonary Arterial Hypertension (Christine Garnett, FDA)**
- **10:35-10:50: Industry Perspective: Model based approaches to support bridging biomarkers (Laurie Conklin, Janssen)**
- **10:50-11:05: Academic Perspective (Thomas Fleming, Univ. of Washington)**

11:05-11:45 am: Case example (Pediatric heart failure)

- 11:05-11:20: Assessing disease similarity: Heart failure in adult and pediatric patients (Daphne Hsu, Albert Einstein College of Medicine)
- 11:20-11:45: Leveraging adult data to support a biomarker extrapolation: Entresto (Ron Portman, Simon Wandel, Guenther Mueller- Velten, Novartis)

11:45 am-1:05pm: Q&A and Panel discussion

Q&A: All speakers

- **Panel discussion:** Daphne Hsu (Albert Einstein College of Medicine) Jialu Zhang (FDA), Norm Stockbridge (FDA), Ron Portman (Novartis), Solange Corriol-Rohou (AstraZeneca), Lynne Yao (FDA), Thomas Fleming (Univ. of Washington), Sudharshan Hariharan (FDA), Robert Nelson (J&J)

1:05-1:15 pm: Christine Garnett (FDA)

Day 2: September 2nd 10am-3:00pm

Session 2: Bayesian techniques in Pediatric Studies

Co-chairs: James Travis (FDA) /John Alexander (FDA)

10:00-10:10 am: Welcome/Introduction (James Travis, FDA)

10:10-11:05 am: Regulatory perspective

- 10:10-10:30: FDA Perspective (Mark Rothmann, FDA)
- 10:30-10:45: EMA Perspective (Andrew Thomson, EMA)
- 10:45-11:05: Industry Perspective (Meg Gamalo, Pfizer)

11:05-12:15 pm: Case examples

- 11:05-11:20: Belimumab approval for pediatric systemic lupus erythematosus (Nicky Best; Anne Hammer, GSK)
- 11:20-11:50: Assessing disease similarity: multiple sclerosis in adult and pediatric patients (Paul Lee, FDA)
- 11:50-12:15: Bayesian approach to support pediatric extrapolation in multiple sclerosis (Marius Thomas and Dieter Häring, Novartis)

12:15-1:00 Break

1:00-2:50 pm: Q&A and Panel discussion

Q&A: All speakers

- **Panel discussion:** Nikolay Nikolov (FDA), Mark Rothmann (FDA), John Lawrence (FDA), Paul Lee (FDA), Robert Nelson (J&J), Forest Williamson

(Lilly), Anna Shmagel (Abbvie), Mathangi Gopalakrishnan (Univ. of Maryland), Andrew Thomson (EMA)

2:50-3:00: Closing remarks (Lynne Yao, FDA)