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

Day 1: September 1st 10am-1:15pm

Opening remarks:

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

10:25-10:55 am: Regulatory perspective
   - 10:25-10:40: FDA Perspective: Pulmonary Arterial Hypertension (Christine Garnett, FDA)
   - 10:40-10:55: Industry Perspective: Model based approaches to support bridging biomarkers (Laurie Conklin, Janssen)

11:10-11:15: Academic Perspective (Thomas Fleming)

11:10-11:25: Assessing disease similarity: Heart failure in adult and pediatric patients (Daphne Hsu, Albert Einstein College of Medicine)
11:25-11:45: Leveraging adult data to support a biomarker extrapolation: Entresto (Ron Portman, Simon Wandel, Guenther Mueller-Velten, Novartis)

11:45 am-1:05pm: Panel discussion
Q&A: All speakers
Panel discussion: Daphne Hsu, Jialu Zhang (FDA), Norm Stockbridge (FDA), Ron Portman (Novartis), Solange Corriol-Rohou (AstraZeneca), Lynne Yao (FDA), Thomas Fleming, Sudharshan Hariharan (FDA)

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)

10:10-10:55 am: Regulatory perspective
  o 10:10-10:25: FDA Perspective (Mark Rothmann, FDA)
  o 10:25-10:40: EMA Perspective (Andrew Thomson, EMA)
  o 10:40-10:55: Industry Perspective (Meg Gamalo, Pfizer)

10:55-12:00 pm: Case examples
  ● 10:55-11:10: Belimumab approval for pediatric systemic lupus erythematosus (Nicky Best; Anne Hammer, GSK)
  ● 11:10-11:35: Assessing disease similarity: multiple sclerosis in adult and pediatric patients (Paul Lee, FDA)
  o 11:35-12:00: Bayesian approach to support pediatric extrapolation in multiple sclerosis (Marius Thomas and Dieter Häring, Novartis)

12:00-1:00 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), Skip Nelson (Janssen), Forest Williamson (Lilly), Anna Shmagel (Abbvie), Mathangi Gopalakrishnan (UMD), Andrew Thomson (EMA)

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