ADEPT 7: Advancing Complex Innovative Trial Designs to Accelerate Drug Development in Pediatric Patients

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

DRAFT AGENDA

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-3pm

10:00-10:10 am

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
  o 10:25-10:40: FDA Perspective: Pulmonary Arterial Hypertension (Christine Garnett, FDA)
  o 10:40-10:55: Industry Perspective: Model based approaches to support bridging biomarkers (Laurie Conklin, Janssen)
  o 10:55-11:10: Academic Perspective (Thomas Fleming)

11:10-11:40 am: Case example (Pediatric heart failure)
  o 11:10-11:25: Assessing disease similarity: Heart failure in adult and pediatric patients (Daphne Hsu, Albert Einstein College of Medicine)
11:25-1140: Leveraging adult data to support a biomarker extrapolation: Entresto (Ron Portman, Simon Wandel, Gunther Mueller-Velten, Novartis)

11:40 am-1:00pm: 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)

1:00-1:05 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), Thomas Fleming (Univ. of Washington), Andrew Thomson (EMA)

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