U.S. FOOD & DRUG ADMINISTRATION

Advancing the Development of Pediatric Therapeutics (ADEPT 6)

Pediatric Clinical Trial Endpoints for Rare Diseases with a Focus on Pediatric Patient Perspectives

FDA White Oak Campus, Building 31 (Great Room), Silver Spring, MD
Tuesday, November 12, 2019

AGENDA		
8:00 – 8:15	**Welcome and Introduction**	**Susan McCune**, FDA
8:15 – 8:35	**Keynote Speaker –** Theme: including pediatric patients from start to finish	**Janet Maynard**, FDA **Scott Espich,** Pediatric Patient Voice
MORNING SESSION: CHOOSING ENDPOINTS		
8:35 – 9:00	**Interview with Pediatric Patients**	
9:00 – 10:00	Endpoints for pediatric patients: Are we asking the right questions? • Focus on fundamentals (age-appropriate endpoints for growth/development) • Rare diseases/Common problems – (endpoints for sleep, pain, etc.)	**Ali Mohamadi**, BioMarin **Heather Adams**, Univ. Rochester **Tonya Palermo**, Seattle Children's Hospital
10:00 – 10:15	**BREAK**	
10:15 – 11:15	• Individualized endpoints • Building bridges to novel endpoints	**Lili Garrard**, FDA **Patroula Smpokou**, FDA **Jin Shei Lai**, Northwestern Univ.
11:15 – 12:00	**Panel Discussion/Q&A**	**Moderator: Pamela Gavin**, NORD
12:00 – 1:00	**LUNCH**	
AFTERNOON SESSION: MEASURING ENDPOINTS		
1:00 – 1:20	**Interview with Pediatric Patients**	
1:20 – 2:20	Data Collection: Old Ways and New • COAs for children • Patient registries – Pediatric Migraine Registry case study	**Courtney Blackwell**, Northwestern **Bradley Marino**, Lurie Children's **Christoph Hornik**, Duke

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2:20 – 2:40	**BREAK**	
2:40 – 3:20	• Real World Evidence • Child- and youth-friendly technology – role of apps & devices	**Robbert Zusterzeel**, Medical Device Innovation Consortium **Lori Crosby**, Cincinnati Children's
3:20 – 4:20	**Panel Discussion/Q&A**	**Moderator: Charles Thompson**, Pfizer
4:20 – 4:30	**WRAP-UP/CLOSING REMARKS – Carla Epps, FDA**	