

FDA/UMD CERSI pJIA Drug Development Workshop
October 2nd, 2019
FDA White Oak, Great Room
Agenda

Introduction	
8:00-8:05	Opening remarks Nikolay Nikolov, MD
8:05-8:20	Regulatory landscape Carolyn Yancey, MD
8:20-8:30	Scientific necessity in the context of pediatric extrapolation of efficacy from adult data Skip Nelson, MD, PhD
8:30-8:40	Parent/Patient Perspective Vincent Delgaizo
Session 1: Disease Similarity in pJIA and RA	
8:40-9:10	Polyarticular JIA: nomenclature, presentation, and relationship to RA Peter Nigrovic, MD
9:10-9:25	Treatment paradigm and landscape of products used in pJIA Dan Lovell, MD
9:25-9:55	Panel Discussion and Q/A Panelists: Carolyn Yancey, Peter Nigrovic, Ricardo Suehiro, Skip Nelson, Dan Lovell, Guy S. Eakin Moderators: Rachel Glaser, MD and Robert Colbert, MD
9:55-10:10am BREAK	
Session 2: Dose Selection and Response Similarity in pJIA and RA	
10:10-10:30	Landscape of pJIA drug development: Regulatory perspective Jianmeng Chen, PhD
10:30-10:45	Exposure and response comparisons: RA vs PJIA Renu Singh, PhD
10:45-11:05	Two Well-Controlled Phase 3 Trials in Patients With Juvenile Idiopathic Arthritis: Challenges and Lessons Learned Jocelyn H. Leu, PharmD, PhD
11:05-11:25	Use of exposure-response information in pediatric drug development Marc Gastonguay, PhD
11:25-12:15	Panel Discussion and Q/A Panelists: Nikolay Nikolov, Hermine Brunner, Mara Becker, Suzette Peng, Vikram Sinha, Chandrahas Sahajwalla, Yaning Wang, Rebecca Rothwell Moderators: Lily Mulugeta and Marc Gastonguay
12:15-1:00pm LUNCH	
Session 3: Confirmation of Efficacy and Assessment of Safety	
1:00-1:20	'Learn & confirm' in JIA: Modeling and Simulations to Support Development and Extrapolation Nael Mostafa, PhD
1:20-1:50	Alternative approaches/trial design: <ol style="list-style-type: none"> 1. Bayesian borrowing of data (adult/historical), Active control trials Rebecca Rothwell, PhD 2. Enrolling pediatric patients into adult trials: Lisa Imundo, MD
1:50 – 2:05	PJIA safety assessment considerations

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	Laura Schanberg, MD
2:05-2:25	Challenges with conducting pediatric trials in pJIA Industry Perspective: Bolanle Akinlade, MD Academia Perspective: Hermine Brunner, MD
2:25-3:15	Panel Discussion and Q/A Panelists: Nikolay Nikolov, Rachel Glaser, Lisa Rider, Lisa Imundo, Vincent Delgaizo, Laura Schanberg, Hermine Brunner, Ricardo Suehiro, Nael Mostafa Moderators: Rebecca Rothwell, PhD and Dan Lovell, MD
3:15-3:30pm BREAK	
Session 4: Moving Forward	
3:30-3:40	Exposure-matching vs confirmation of efficacy: Pros, cons, and remaining uncertainties Nikolay Nikolov, MD
3:40-4:35	Panel Discussion and Q/A Panelists: Nikolay Nikolov, Dawn Territo, Laura Schanberg, Mara Becker, Hermine Bruner, Issam Zineh, Nael Mostafa, Vikram Sinha Moderators: Lynne Yao, MD and Lisa Rider, MD
4:35-4:55	Summary Remarks Session Chairs
4:55-5:00	Closing Remarks Nikolay Nikolov, MD