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

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| **Introduction** | |
| 8-8:05 | **Opening remarks**<br>Nikolay Nikolov, MD |
| 8:05-8:20 | **Regulatory landscape**<br>Carolyn Yancey, MD |
| 8:20-8:30 | **Scientific necessity in the context of pediatric extrapolation of efficacy from adult data**<br>Skip Nelson, MD, PhD |
| **Session 1: Disease Similarity in pJIA and RA** | |
| 8:30-9:00 | **Polyarticular JIA: nomenclature, presentation, and relationship to RA**<br>Peter Nigrovic, MD |
| 9:00-9:15 | **Treatment paradigm and landscape of products used in pJIA**<br>Dan Lovell, MD |
| 9:15-9:45 | **Panel Discussion and Q/A**<br>**Panelists**: Carolyn Yancey, Peter Nigrovic, Ricardo Suehiro, Skip Nelson, Dan Lovell, Guy S. Eakin<br>**Moderators: Rachel Glaser, MD and Robert Colbert, MD** |
| **9:45-10:00am BREAK** | |
| **Session 2: Exposure and Response Similarity in pJIA and RA** | |
| 10:00-10:20 | **Landscape of pJIA drug development: Regulatory perspective**<br>Jianmeng Chen, PhD |
| 10:20-10:35 | **Exposure and response comparisons: RA vs PJIA**<br>Renu Singh, PhD |
| 10:35-10:55 | **Two Well-Controlled Phase 3 Trials in Patients With Juvenile Idiopathic Arthritis: Challenges and Lessons Learned**<br>Jocelyn H. Leu, PharmD, PhD |
| 10:55-11:15 | **Use of exposure-response information in pediatric drug development**<br>Marc Gastonguay, PhD |
| 11:15-12:05 | **Panel Discussion and Q/A**<br>**Panelists:** Nikolay Nikolov, Hermine Brunner, Mara Becker, Suzette Peng, Vikram Sinha, Chandrahas Sahajwalla, Yaning Wang, Rebecca Rothwell<br>**Moderators: Lily Mulugeta and Marc Gastonguay** |
| **12:05-1:00pm LUNCH** | |
| **Session 3: Confirmation of Efficacy and Assessment of Safety** | |
| 1:00-1:20 | **Modeling and Simulation to Support Extrapolation**<br>Nael Mostafa, PhD |
| 1:20-1:50 | **Alternative approaches/trial design:**<br>1. **Bayesian borrowing of data (adult/historical), Active control trials**<br>Rebecca Rothwell, PhD<br>2. **Enrolling pediatric patients into adult trials**:<br>Lisa Imundo, MD |
| 1:50 – 2:05 | **PJIA safety assessment considerations**<br>Laura Schanberg, MD |

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| 2:05-2:25 | **Challenges with conducting pediatric trials in pJIA**<br>Industry Perspective: **Bolanle Akinlade, MD**<br>Academia Perspective: **Hermine Brunner, MD** |
| 2:25-3:15 | **Panel Discussion and Q/A**<br>**Panelists:** Nikolay Nikolov, Rachel Glaser, Lisa Rider, Lisa Imundo, Vincent Delgaizo, Laura Schanberg, Hermine Brunner, Ricardo Suehiro, Nael Mostafa<br>**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**<br>Nikolay Nikolov, MD |
| 3:40-4:35 | **Panel Discussion and Q/A**<br>**Panelists:** Nikolay Nikolov, Dawn Territo, Laura Schanberg, Mara Becker, Hermine Bruner, Issam Zineh, Nael Mostafa, Vikram Sinha<br>**Moderators: Lynne Yao, MD and Lisa Rider, MD** |
| 4:35-4:55 | **Summary Remarks**<br>Session Chairs |
| 4:55-5:00 | **Closing Remarks**<br>Nikolay Nikolov, MD |