

## **Quantitative Assessment of Assumptions to Support Extrapolation of Efficacy in Pediatrics:**

### **Final Report**

#### **University of Maryland Center (UMD) of Excellence in Regulatory Science and Innovation (M-CERSI)**

#### **Science Exchange and Conference Grants**

The program, Quantitative Assessment of Assumptions to Support Extrapolation of Efficacy in Pediatrics, was held on June 1, 2016 at the Food and Drug Administration (FDA) White Oak Campus from 8:00am to 5:00pm.

The workshop had two objectives: 1) to discuss quantitative and qualitative approaches for verifying assumptions pertaining to disease and therapeutic response similarity between adults and children, and 2) to explore the role of alternative approaches in the assessment of extrapolation assumptions in pediatric product development.

The workshop commenced with presentations by speakers from the FDA, industry, and academia. Opening remarks were made by Dr. Janet Woodcock, Director of Center for Drug Evaluation and Research at the FDA. Following her presentation, the first half of the program was focused on the use of extrapolation as an alternate for conducting adequate and well controlled efficacy trials in children. Several case examples in partial onset seizures, inflammatory bowel diseases, and poly-articular juvenile idiopathic arthritis were shared to exemplify quantitative and qualitative approaches to assessing disease and therapeutic response similarity between adults and pediatrics. The presentations were followed by a panel discussion, which included representation from the European Medicines Agency. The afternoon was focused on the use of disease models, clinical trial simulation, and Bayesian approaches in assessing extrapolation assumptions. Again, some case examples were shared and the presentations were followed by a panel discussion. The workshop concluded with closing remarks from Dr. Issam Zineh, Director of the FDA's Office of Clinical Pharmacology, who summarized the workshop and provided some future direction for the use of quantitative approaches in pediatric drug development.

The program was advertised to the American College of Clinical Pharmacology (ACCP), Pediatric Pharmacy Advocacy Group (PPAG), International Society of Pharmacometrics (ISoP), FDA, American Society for Clinical Pharmacology and Therapeutic (ASCPT), and UMD School of Pharmacy. The program was a success with over 800 participants which included in-person and online participants.

A majority of the budget was spent on travel reimbursement for the industry speakers. The planning committee included: Joga Gobburu, PhD from the University of Maryland School of Pharmacy; Lily Mulugeta, PharmD; Robert Nelson, MD; Kevin Krudys, PhD; Gil Burkhart, PharmD; Andrew Mulberg, MD; Nitin Mehrotra, PhD; Lynne Yao, MD; Gregory Reaman, MD; Barbara Buch, MD; Vasum Peiris, MD; Vikram Sinha, PhD; and representatives from the Office of Regulatory Science and Innovation (ORSI) at the FDA.