Application of pharmacometrics in children with rare disease-experiences and future applications

Co-Chairs
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Description
Rare diseases are paradoxically common, collectively affecting around 25 million people in U.S. and have a huge impact on public health. Nearly 50-75% rare diseases begin in childhood, continue throughout life, are disabling and life threatening. Therefore, these pediatric disorders deserve special priority for improved health care and advancement of medicine. Current challenges for development of novel therapies for rare diseases include small number of patients, especially children and incomplete knowledge regarding mechanism of pathogenesis. In recognition of these challenges, US FDA has provided regulatory and financial incentives aimed at stimulating investment in orphan drugs to treat rare diseases.

Pharmacometrics is a bridging science applying quantitative analysis to characterize physiology, pharmacology, and disease progression. Pediatric pharmacometric strategies are now increasingly applied to aid efficient drug development and regulatory decision making. The proposal for this session is to discuss about the opportunities and challenges of drug development for children with rare disease. Experts from pharmaceutical industry, consulting company and agency will present the case studies from complimentary perspectives to highlight the ongoing pharmacometrics efforts covering various components of pediatric drug development in rare disease, from optimizing study design, to selecting safe and effective dosing regimen(s) across the pediatric age spectrum.

Learning Objectives

The challenges and opportunities of drug development for children with rare diseases

Model-informed drug development to bring new medicines to children with rare diseases

Understand the role of pharmacometrics to facilitate dose selection for children with rare diseases.
Session Speakers and Presentations

Lutz Harnisch  -  Getting the dose right, first! Challenges to pharmacometric approaches in children with rare diseases.

Xiang Gao  -  Optimizing modeling and simulation to bridge data for efficient pediatric drug development in rare diseases

Karen Rowland Yeo  -  Application of PBPK modelling for rationalizing dose selection in pediatric rare diseases

Luning (Ada) Zhuang  -  Dosage considerations for FDA approval of canakinumab in pediatric rare diseases

Optimizing modeling and simulation to bridge data for efficient pediatric drug development in rare diseases

Application of PBPK modelling for rationalizing dose selection in pediatric rare diseases

Presentation: Dosage considerations for FDA approval of canakinumab in pediatric rare diseases