**Subteam: Pediatric and Small Population Drug Development**

**Leader Information**

 Margaret “Meg” Gamalo (Eli Lilly & Co) gamalo\_margaret@lilly.com

**Subteam Overview**

We are a group of individuals from pharma, academia, and regulatory agency, within the Bayesian Scientific Working Group (BSWG), with special interest in pediatrics and therapeutic orphans.

Therapeutic orphans and pediatrics share a common problem, both involve small populations prohibiting investment in drug development as well as creating barriers to both sizing clinical trials with sufficient power, recruitment, and research infrastructure. They also share the same need for action - there are over 7000 rare disease but only a small fraction of those have approved orphan medicines and only about 20 percent of drugs approved by the FDA are labeled for pediatric use. That being said, the current scenario calls for cross-discipline efforts to spur research and one where statistics can be a valuable tool to balance feasibility and informativeness through the use of efficient and innovative trial designs. While the subteam is within BSWG, we do NOT limit our projects to Bayesian methodology only.

Objectives of the subteam:

1. To explore statistical methodology that can be applicable in the design of analysis of clinical trials with particular interest in applying Bayesian methodology.
2. To illustrate and provide advice on best practices that could be used by Pharma and CRO statisticians in designing trials for pediatric and orphan therapeutics.
3. To collaborate with pharma, academia and regulatory bodies to exchange problems/issues as well as possibilities where consensus in solutions can be made
4. To disseminate information on research and best practices to broader scientific community as well as to engage in education efforts through conferences, workshops and seminars.

**Members**: Laura Thompson (FDA/CDRH), Brad Carlin (U. Minnesota), Ram Tiwari (FDA/CDER), Andy Kenwright (Roche), Naomi Givens (GSK), Guochen Song (Biogen), Simin Baygani (Eli Lilly), Xin Zhao (Johnson & Johnson), Junjing “Jane” Lin (AbbVie), H. Amy Xia (Amgen), Freda Cooner (FDA/CDER), Aijun “Angela” Gao (Chiltern), Shiling Ruan (Novartis), Mathangi Golapakrishnan (U Maryland), Clara Domingues-Islas (MRC/GSK), Junshan Qiu (FDA/CDER), Yodit Seifu (Ferring), Karen Price (Eli Lilly), Jennifer Clark (FDA/CDER)

**How to join our cause**: Please send email to Meg Gamalo (gamalo\_margaret@lilly.com) or any of the members of the subteam.

**Ongoing Projects**

1. Innovative and Efficient Trial designs

Members: Meg Gamalo, H. Amy Xia, Freda Cooner, Aijun “Angela” Gao , Shiling Ruan

1. Quantitative Assessment of Similarity to Support Extrapolation

Members: Mathangi Golapakrishnan , Clara Domingues-Islas, Jane Lin, Meg Gamalo, Junshan Qiu, Yodit Seifu

1. Randomization in Small Populations

Members:

1. Outreach and Communication

Members: Yodit Seifu, Freda Cooner, Meg Gamalo, Karen Price

**Resources**

**Statistical Modeling for Bayesian Extrapolation of Adult Clinical Trial Information in Pediatric Drug Evaluation**, Margaret Gamalo-Siebers, Jasmina Savic, Cynthia Basu, Xin Zhao, Mathangi Gopalakrishnan, Aijun Gao, Guochen Song, Simin Baygani, Laura Thompson, H. Amy Xia, Karen Price, Ram Tiwari, Brad Carlin

**Small Population Trial Design Considerations,** Freda Cooner, Ph.D. and Ram Tiwari, Ph.D., Office of Biostatistics, Office of Translational Sciences, CDER/FDA

**European Medicines Agency: EMA Paediatric Medicines Office and PDCO Enpr-EMA**, Irmgard Eichler