Pediatric Formulation : Global Alignment and Science Advancement

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IQ Symposium 2014 – “Pre-competitive Collaboration for Innovation”

Pediatric Formulation Working Group Members

Leadership

- Rob Ju (AbbVie)
- Julia Gao (BMS)
- Beth Galella (BMS)
- Bob Ternik (Eli Lilly)
- Karen Thompson (Merck)
- Trupti Dixit (Takeda)

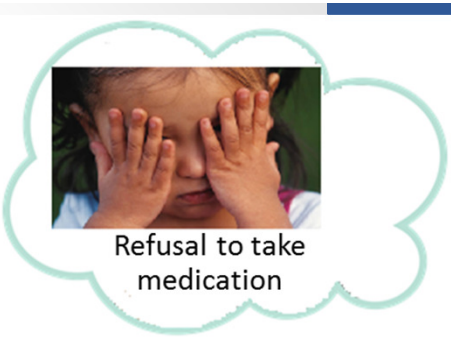
Team members

- Mei Khong (AbbVie)
- David Tan (AbbVie)
- Ji Zhou (AbbVie)
- Fernando Alvarez-Nunez (Amgen)
- Richard Creekmore (AstraZeneca)
- Terri Albarano (Baxter)
- Anjali Agrawal (BI)
- Vijay Naringrekar (Celgene)
- Cindy Ciliberto (Endo)
- Lorrene Buckley (Eli Lilly)
- Nats Rajagopalan (Eli Lilly)
- Daniel Schaufelberger (J&J)
- Steve Wiet (J&J)
- John Heimlich (Pfizer)
- Alpa Parikh (Teva)
- Eleni Dokou (Vertex)
- Meghna Israni (Vertex)
- Phil Bransford (Vertex)
- Meghan Johnston (Vertex)
- Jim Polli (U of Maryland)

New members since 2Q14

- *Z. Jane Li* (BI)
- Mary Tanenbaum (BI)
- Stephanie A Sweetana (Lilly)
- Siddhesh Patil (Takeda)

Background/ Problem Statement



- Inconsistent and unclear global regulatory requirements
- Lack of fundamental understanding of key product performance, including palatability, swallowability, and taste assessment
- Stringent safety criteria for excipients due to lack of safety information or perceived toxicity concerns in the target population
- Challenges to achieve dose flexibility, dose uniformity and convenience at the same time
- Unique ADME profiles in children and thus BA/BE criteria

Mission and Vision

- To serve as the primary contact for pediatric formulation sciences in the U.S.
- To promote global collaboration and advocate for global harmonization for pediatric product requirements
- To be a catalyst for advancing science & technology of pediatric products
- To share best practices, publish information, provide mechanisms for collaboration and learning

Accomplishments

Assessed EuPFI STEP Excipient Database

Comments on a key EMA guideline on pharmaceutical development of paediatric use

EuPFI f2f meeting

1H 2013

Sub-teams formed

Vision & Mission

Rolled out a global survey

2H2013

IQ signed Collaboration Agreement with EuPFI

Provided common excipients to STEP Excipient Database

Survey results presented in IQ CMC Summit and EuPFI annual meeting

Several initiatives with EuPFI

TC with FDA

2014

Impact

- Share our survey results to guide global alignment
  - IQ, EuPFI annual meeting (Sep ‘14), GRiP webinar (Jan ‘15)
  - Key areas with significant gaps:
    - Palatability
    - Age-appropriate formulations
    - Excipients
    - Biopharmaceutics
    - Mis-alignment of global regulatory requirements
- Survey medical professionals to address gaps in pediatric physiology (e.g., size and volume of dosage forms) and to inform stakeholders through a whitepaper
- As opportunities arise, collaborate with CPLG & PSLG in the areas of biopharmaceutics and excipient use, respectively

Impact - continued

- Advocate for global alignment and partner with the leading consortium in pediatric formulation (EuPFI)
  - Gathering feedback received from EMA/FDA on PIP/PSP submitted by member companies and provide findings and constructive feedback to regulatory agencies for better consistency
  - Planning a 2016 joint IQ/EuPFI/FDA workshop
- US Voice and Alignment
  - IQ has become a significant voice in the US for pediatric formulation development
  - Continuing to align US effort with other organizations such as USPFI