

“Pediatric Formulation Development Challenges of Today and Strategies for Tomorrow”

June 18-19, 2019

Session #4 - Regulatory/Industry

Industry Perspective: Presentation



Globalization & Harmonization **Beyond US and EU?**

US and EU

- Regulations & Incentives established
- IQ Consortium (US), Pediatric Working Group; AAPS
- European Paediatric Formulation Initiative (EuPFI)



Other regions?

- Japan, China; Countries/regions involved in clinical studies?
- India – plans for “Indian Paediatric Formulation Initiative” (IPFI)
- Regions where “Neglected Tropical Diseases” affect large numbers of children
- **WHO** initiatives:

Pre-Qualification Process – <https://extranet.who.int/prequal/information/manufacturers>

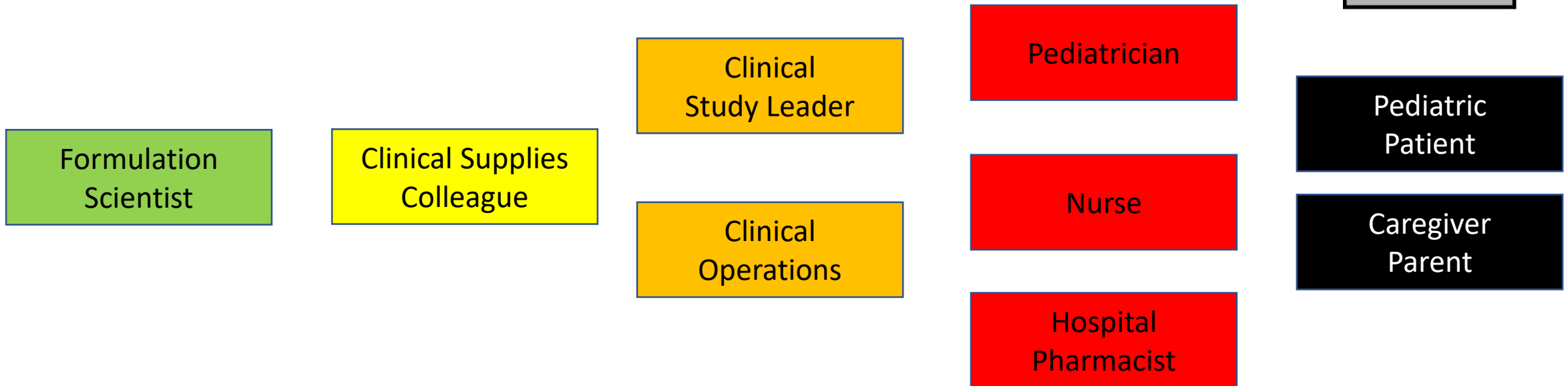
Paediatric Development Tool Kit (anti-HIV), Module 5: Acceptability:

https://globalhealthtrainingcentre.tghn.org/site_media/media/medialibrary/2018/07/WHO_Research_Toolkit_Module_5.pdf

Anybody doing GLOBAL HARMONIZATION?

Patient-Centric Design for the Formulation Scientist

Challenge: “distance” formulation scientist <-> patient
Solutions? -> breakout session



New Kids on the Block?

Private-Public Partnerships (PPP) as New Partners Developing Pediatric Products

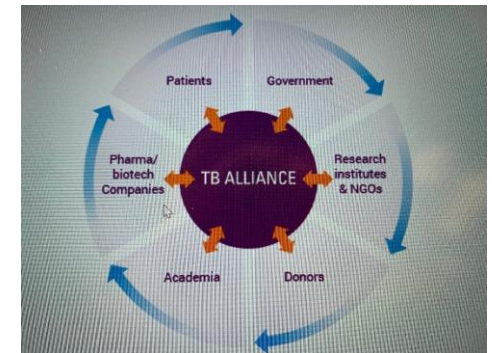
Bill & Melinda Gates Medical Research Institute

Focus on access and affordability (CMC) www.gatesmri.org

Tuberculosis: TB Alliance

A not-for-profit Product Development Organization developing next generation products to treat multidrug-resistant TB

www.tballiance.org



Schistosomiasis: Pediatric Praziquantel Consortium

“Our mission is to develop, register and provide access to a suitable pediatric praziquantel formulation for treating schistosomiasis in preschool-age children”

www.pediatricpraziquantelconsortium.org

Synergies - Paradigm shift?

Big Data, Artificial Intelligence (AI) & Machine Learning

- Everybody in Health Care talks about it
- Universities offering (on-line) classes; companies announcing collaborations
- Already established in medicinal chemistry
- Potential in image analysis, e.g. histopathology

- Pediatrics

- “Can AI better diagnose childhood diseases?”
- Outcomes? Real-world evidence?
- **Are we ready to explore AI for pediatric formulation/product development?**

