

Expectations for the 2019 Pediatric Formulations Workshop Outcomes

Pediatric Formulation Development: Challenges of Today and Strategies for Tomorrow

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Some Drivers/Catalysts bringing us together for advancing pediatric formulations

- 1) Area of unmet need and of significant global impact as evident in the growing needs for:
 - Accessible age-friendly pediatric medicines
 - Acceptable drug products for ensuring adherence to the prescribed treatment
- 2) Complexity of advancing pediatric formulations and dosage forms requires
 - integrated multidisciplinary/multispecialty and multidimensional collaborations for framing and solving problems, and
 - for generating/sharing/leveraging critical knowledge for sustained benefit
- 3) Robust, and long term solutions require
 - Building partnerships and
 - Advancing integrated communities for shared expectations through better risk awareness, risk balancing and risk alignment

The outline of my talk

- 1) Some Drivers/Catalyst for this workshop
- 2) Outline
- 3) Workshop goals
- 4) Catalysts for change
- 5) Bird's eye view of the program
 - The two-day program
 - Points to consider, ideas generating ideas
- 6) Deliverables: During and after the workshop

Workshop Goals in the Program

- 1) Sharing knowledge (e.g., practices, issues, and challenges in pediatric drug development)
- 2) Exchange of ideas and experiences in pediatric drug development for age-appropriate formulations
- 3) Clarifying data needs for integrated risk assessment and identifying best practices for generating data for global use.

And

Be present and participate and make it happen!

Breakout sessions and panel discussions
are approx. 80% of the program.

Catalysts for Change: Impact on Global Health Care

For 2015

Children (under 19 years of age):

In the USA: Approx. **25%** of the US population (322M)

And

In the world: Approx.

34.5% of the world population (7.35 Billion)



Projections for 2050

Children (under 19 years of age):

In the USA: Approx. **25%** of the US population (389 M)

And

In the world: Approx. **28%** of the world population (9.7 Billion)

Possibilities?: Positive impact of access to age-friendly medicines and acceptability of drug products (i.e. adherence to treatment)

Advancing Development of Pediatric Drug Products Requires Integrated Efforts

1) Patient and drug product understanding/knowledge

- Therapeutic needs of the targeted patient population (heterogenous population from preterm to young adults)
- Getting to the right drug, right dose, right dosage form/formulation for the patient
- Manufacturing technology for commercial production of a drug product (with reliable/consistent performance, and acceptable stability and more (e.g. accessible and acceptable by the targeted patient population))
- “Knowledge” tools: creativity/resourcefulness in generating/sharing/leveraging data and its translation into “actionable knowledge”.

Advancing Development of Pediatric Drug Products Requires Integrated Efforts (contd.)

2) Ability to distill/translate knowledge for optimizing the drug product and the patient and drug product interface

– Relies on effective multidisciplinary, multispecialty and multidimensional collaborations in areas such as:

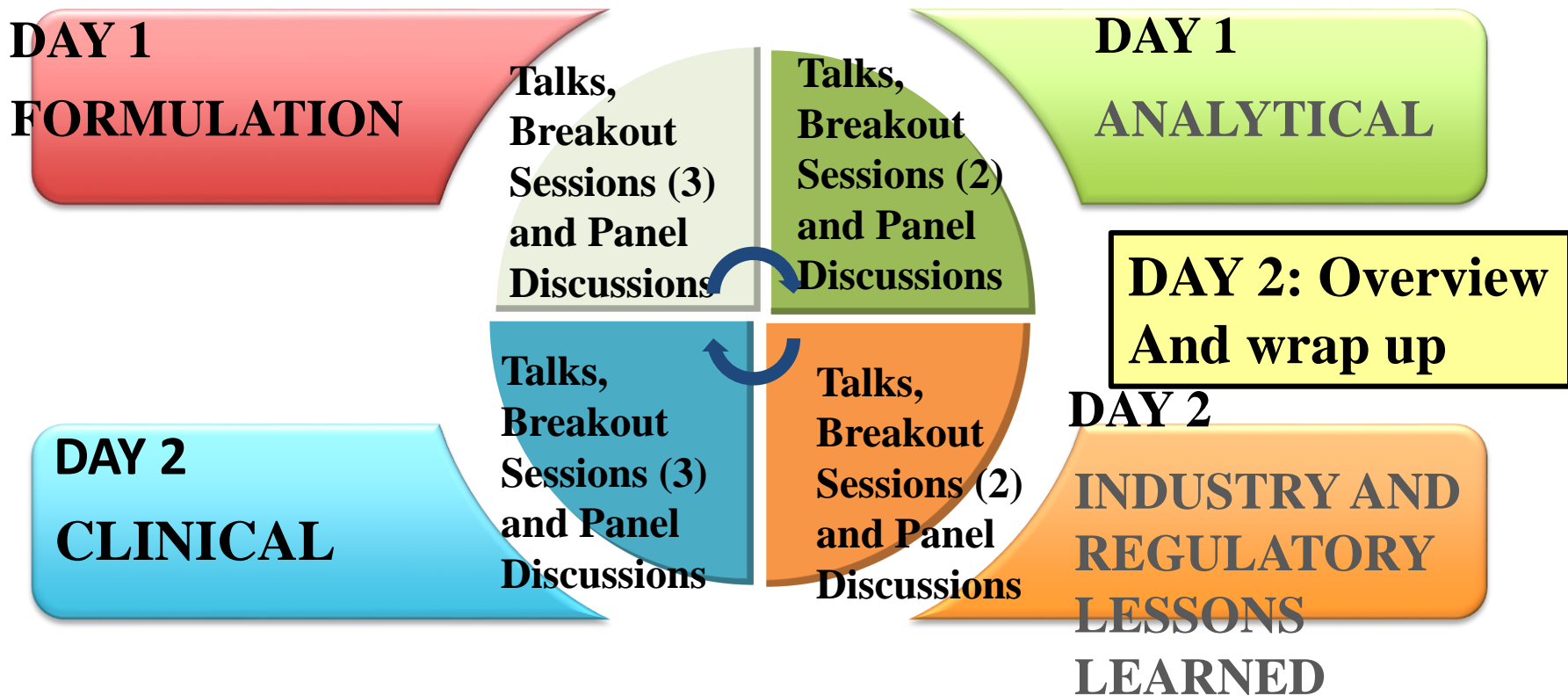
- + Formulations
- + Analytical Methodology
- + Clinical
- + Sharing/leveraging Lessons Learned

Efforts for expanding possibilities for shared risk appreciation and aligning expectations

Building on collaborations, developing partnerships and expanding possibilities for shared risk appreciation and alignment for agreeing on expected outcomes

- Learning from each other, sharing/leveraging knowledge for risk awareness/assessment/alignment
- Developing a roadmap for Integrated risk assessment/alignment
- Creating a “space” for meaningful collaborations and for advancing creative and innovative approaches for advancing development of pediatric formulations

Bird's Eye View of Our Workshop



Some things to consider/explore during the workshop: Ideas generating ideas

For advancing pediatric formulations

- What are the areas that can be of greatest return?
 - What resources are needed?
 - What may be possible advances as next steps (strategies for today and tomorrow?)
- What is needed to accomplish the next best steps?
- What would “resourcefulness” look like?

Share your insight/experience/expertise

- Breakout discussions, panel discussions and
- Add notes/comments to the idea canvas (the innovation) poster

Deliverables

- During the workshop
 - Discussions, shared ideas/notes
 - Proposed future activities
- After the workshop
 - Publications
 - Staying engaged and connected for ongoing and future efforts

Perceived or Intrinsic Risk?



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