BPCA Prioritization – Identification of Knowledge Gaps in Pediatric Drug Development

OMB # 0925-0766

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In preparation for the 2020 BPCA Annual Meeting December 14-15, 2020, we would like your feedback on therapeutic gaps in pediatric drug development in order to inform the 2021 prioritization process. Please complete the following survey to share your input. We look forward to your participation at the meeting!

Name/Contact Details

Name

Institution

Email Address

Specialty Area/Therapeutic Area Focus

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1. Advancing Clinical Trial Design

- Validated endpoints in a non-static state
- New drug development and new therapeutic uses

Please provide your feedback on this topic below:

What are the remaining questions in pediatric trial designs? Where is the innovation happening?

What guidelines do you currently use in selecting the correct trial design?

How can we improve the success of clinical trial design in pediatric drug development clinical trials?

What components do you think are integral to building an effective framework as it relates to advancing clinical trial design?

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2. PK Modeling that Informs Dosing

- More research/training on extrapolation from adult studies
- Need a standard for PK modeling for drug development, safety, trials design
- Need for comprehensive comparative data across various therapeutic areas

Please provide your feedback on this topic below:

Is extrapolation from adult data possible in your disease? If not, what research (i.e., outcome measures, endpoints) remains to be done for extrapolation to occur?

If extrapolation is not possible in your area of research, what tools, designs, or research questions remain in your specialty?

What components do you think are integral to building an effective framework as it relates to PK modeling?

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3. Pharmacoepidemiology studies

- Improved process for pharmacovigilance (therapeutic drug monitoring)
- Developing toxicity profiles across various therapeutic areas

Please provide your feedback on this topic below:

Are there existing pharmacovigilance databases that are pediatric focused? If so, how do we gather the data to accomplish the above?

How can pharmacoepidemiology be used to advance research in "omics?" Who are the key players in this type of research?

What components do you think are integral to building an effective framework as it relates to pharmacoepidemiology studies?

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4. Pediatric Friendly Formulations

Please provide your feedback on this topic below:

How do we attract industries to this market?

To your knowledge, where are the existing needs in the development of pediatric friendly formulations?

What components do you think are integral to building an effective framework as it relates to pediatric friendly formulations?

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5. Systems Pharmacology

- Need for more research on the ontogeny of disease and treatment response including: transporters, biomarkers, natural history data
- Need for more research on the impact of ontogeny on "omics"

Please provide your feedback on this topic below:

To your knowledge, what research currently exists in the area of 'quantitative' systems pharmacology in pediatrics? Who is conducting this type of research?

Are there existing knowledge gaps in the above areas in your specialty?

What advantages or disadvantages have you encountered in targeted therapies in your research?

What components do you think are integral to building an effective framework as it relates to systems pharmacology?

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6. Biomarkers Research

Please provide your feedback on this topic below:

In your opinion, what do you define as the most 'relevant' biomarkers research needed in pediatric drug development?

What components do you think are integral to building an effective framework as it relates to biomarker selection?

Thank you for your feedback! Your input is vital to the successful implementation of the NIH BPCA program.
If you have additional questions or comments, please send email to bpca@infinityconferences.com.