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Discussion

Facilitating Peds-First Drug Development Approach

Discussion Session

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Current Status

- Since 1997, there have been legislative and regulatory approaches to address pediatric product development.
  - Best Pharmaceuticals for Children Act (BPCA)
  - Pediatric Research Equity Act (PREA)
- Both acts were made permanent in 2012, with modifications, in Title V of the Food and Drug Administration Safety and Innovation Act (FDASIA).

Progress has been made!

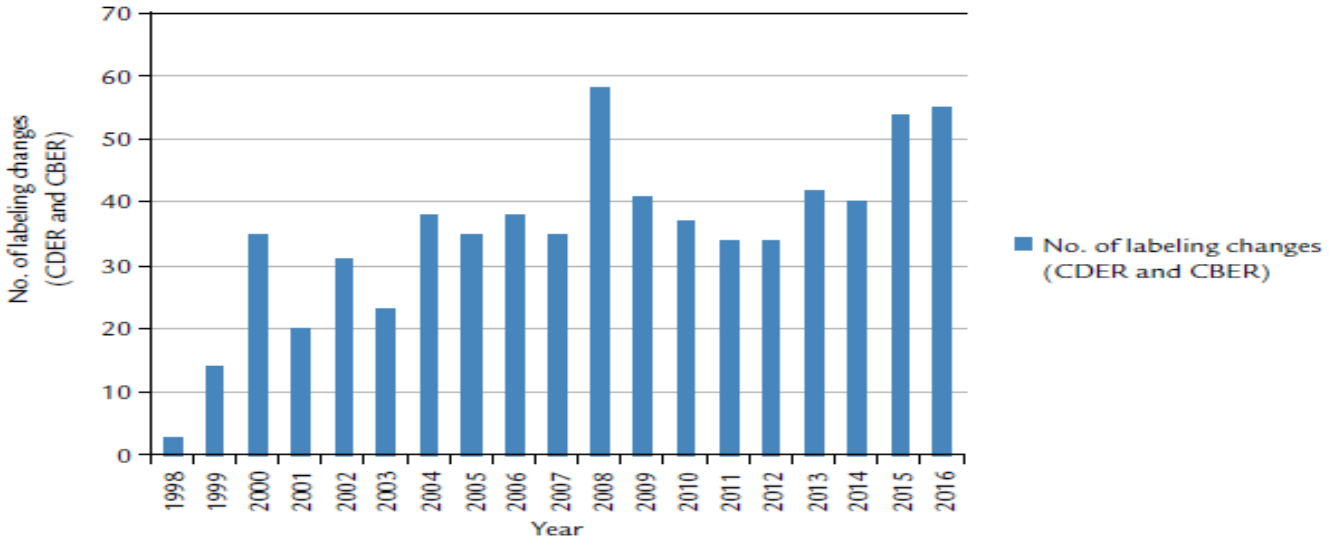

Figure 1. Pediatric labeling changes in the United States since the passage of pediatric-specific legislation, including the Best Pharmaceuticals for Children Act, the Pediatric Research Equity Act, and the Pediatric Rule. Center for Biologics Evaluation and Research and Center for Drug Evaluation and Research regulated biologics with pediatric labeling changes before September 27, 2007, are not included in the US Food and Drug Administration (FDA) database. Data are from the FDA New Pediatric Labeling Information Database.[24]

Despite this, studies in neonates, infants, childhood rare diseases, and disorders that are routinely diagnosed in childhood remain a challenge

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Areas of Opportunity

- Currently, pediatric trials are often after adult trials have completed
  - Disorders that primarily occur in children
  - Disorders that typically occur early in childhood but are not being investigated
  - Unique safety/toxicity concerns
  - Dosing considerations including formulation types
- Pediatric drug development requires a complex set of skills
- Some diseases have high placebo response rate
- Need for skilled researchers
  - Able to identify subtle, but meaningful changes
- Primarily, children who are geographically close to trial sites or willing/able to travel long distances consistently can participate

Thoughts for Discussion

- How can we change the research paradigm to change the trajectory of disease progression and long-term outcomes?
- How do we work to change things so that these disorders are not considered too risky or not financially viable to pursue?
- What are the biggest obstacles?
- What would be the next steps?

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