

# Pediatric Therapeutics Development in the 21<sup>st</sup> Century: It's Not Your Mother's Clinical Trial

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- I have no financial relationships to disclose relating to this presentation
- The views expressed in this talk represent my opinions and do not necessarily represent the views of FDA
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# Brief History of Pediatric Drug Development

1994

- Pediatric Labeling Rule
- Introduces concept of Pediatric Extrapolation

1997

- First incentives laws for pediatric drug development passed
- Becomes Best Pharmaceuticals for Children Act (BPCA) in 2002

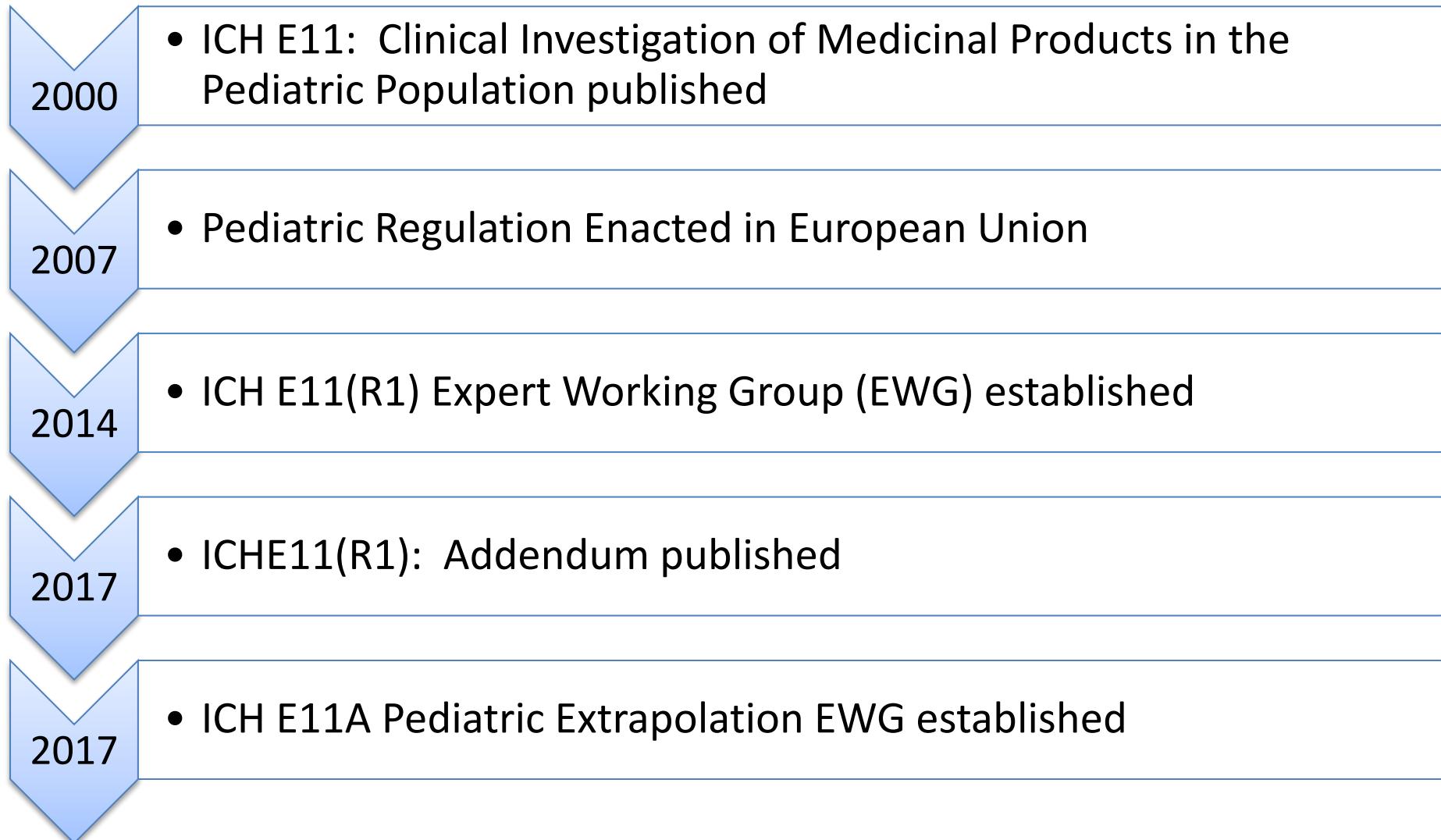
1998

- First requirement for pediatric drug development promulgated by FDA
- Becomes Pediatric Research Equity Act in 2003

2000

- ICH E11: Clinical Investigation of Medicinal Products in the Pediatric Population published

# Brief History of Pediatric Drug Development

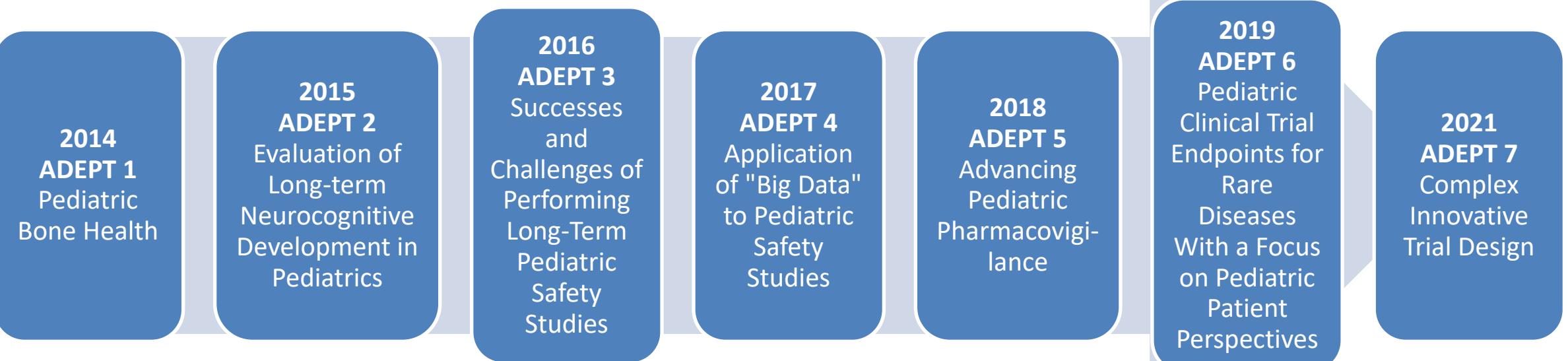


# Pediatric Therapeutics Development in the 21st Century



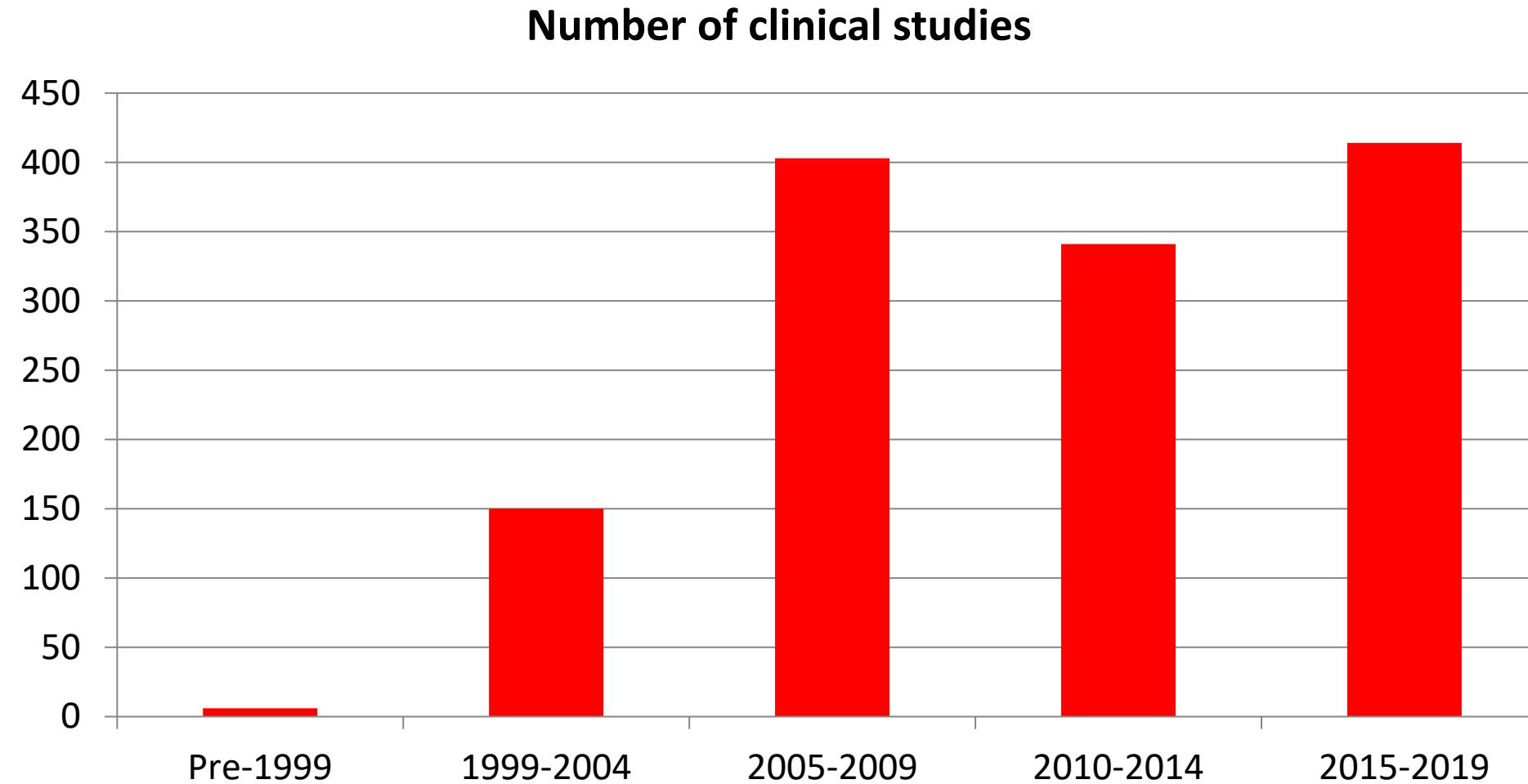
- Increased regulatory and scientific alignment
- Increased application of pediatric extrapolation
- Increased efficiency in clinical trial operations
- Increased global collaboration

# Advancing the Development of Pediatric Therapeutics (ADEPT) Workshops



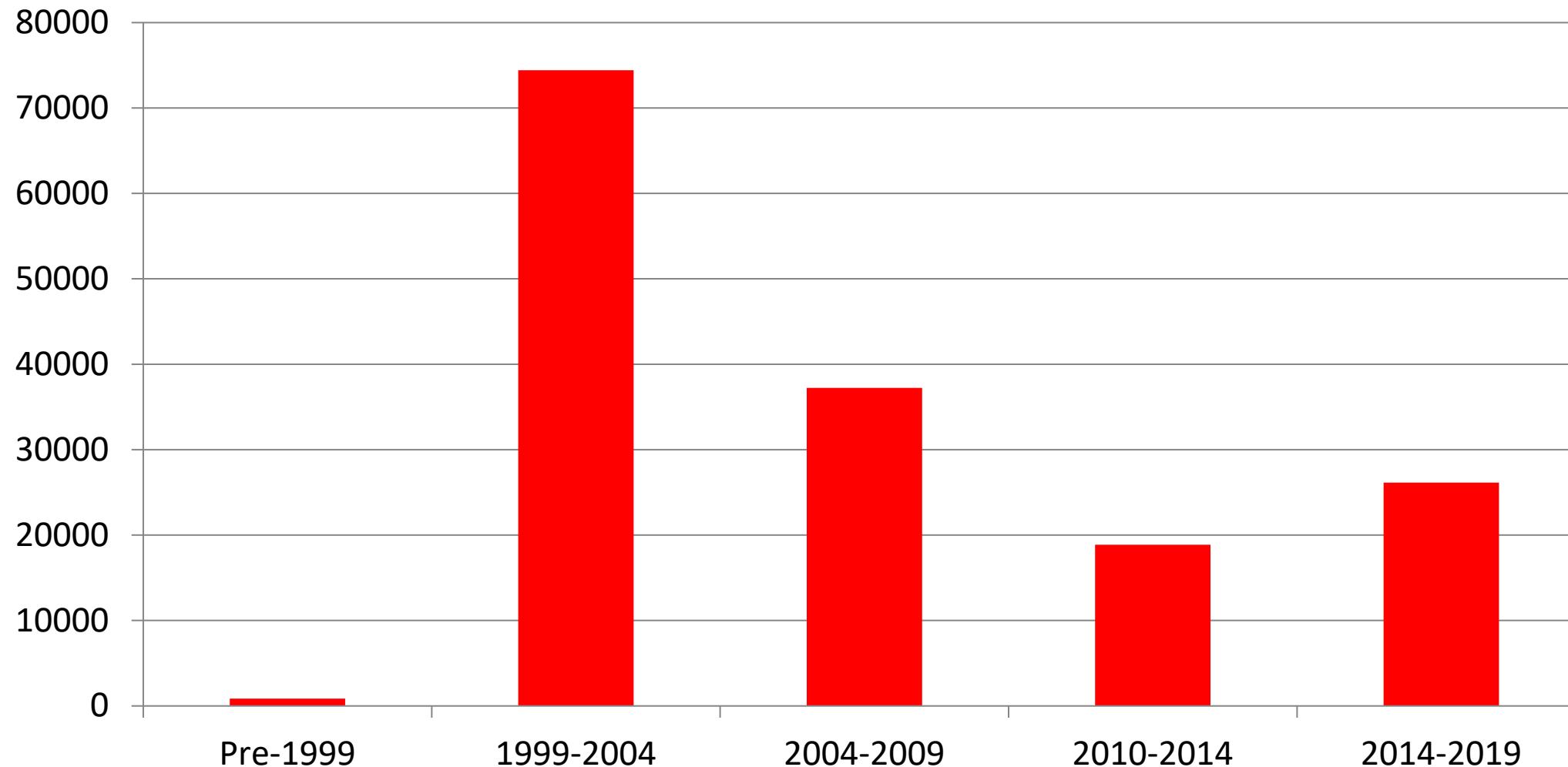
Provide a platform for scientific exchange among the FDA, other global health authorities, patient advocates, and drug developers to increase efficiency and success of pediatric therapeutics development

# Number of Studies Completed under BPCA and PREA





# Number of children enrolled in trials under BPCA and PREA (n=152,675)



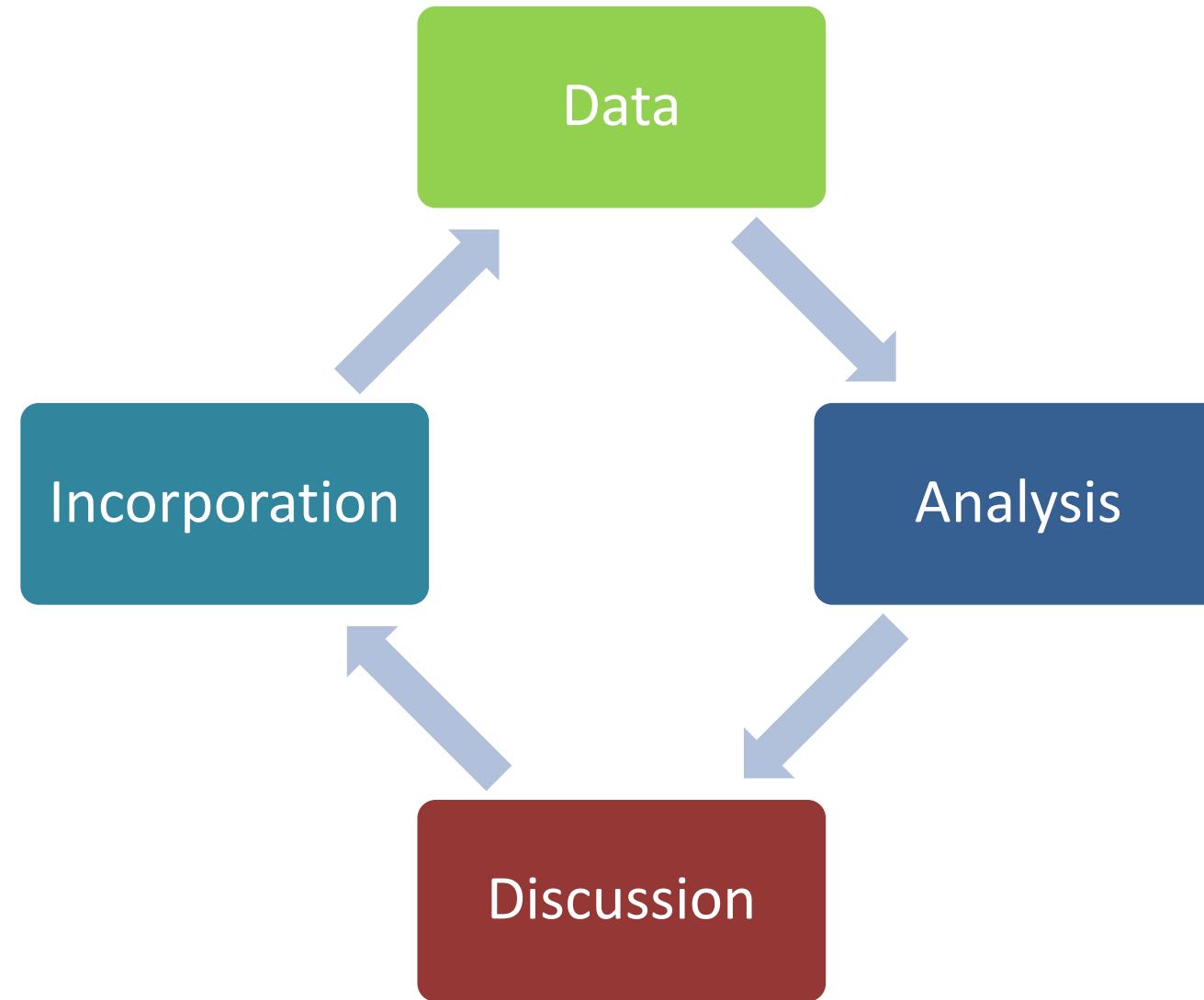
# Advances in the Application of Pediatric Extrapolation

Consideration of  
all existing  
relevant data

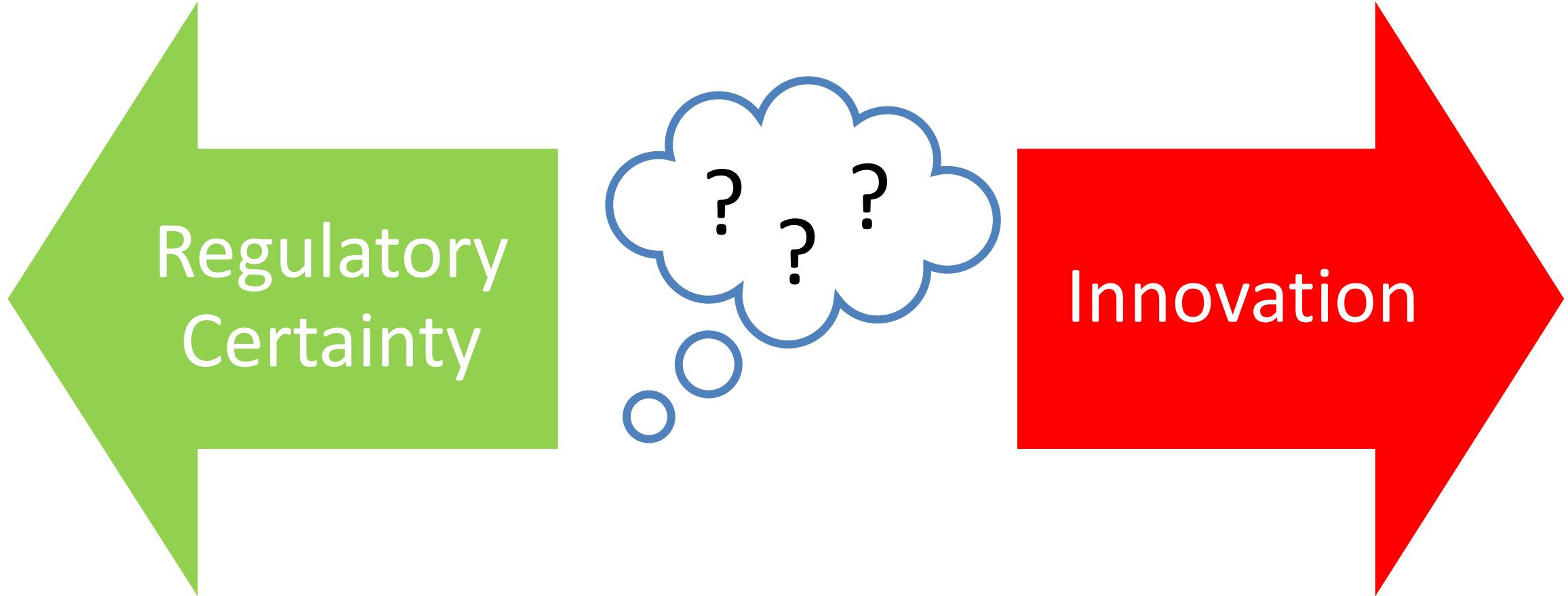
Utility of modeling  
and simulation

Application of  
innovative trial  
designs

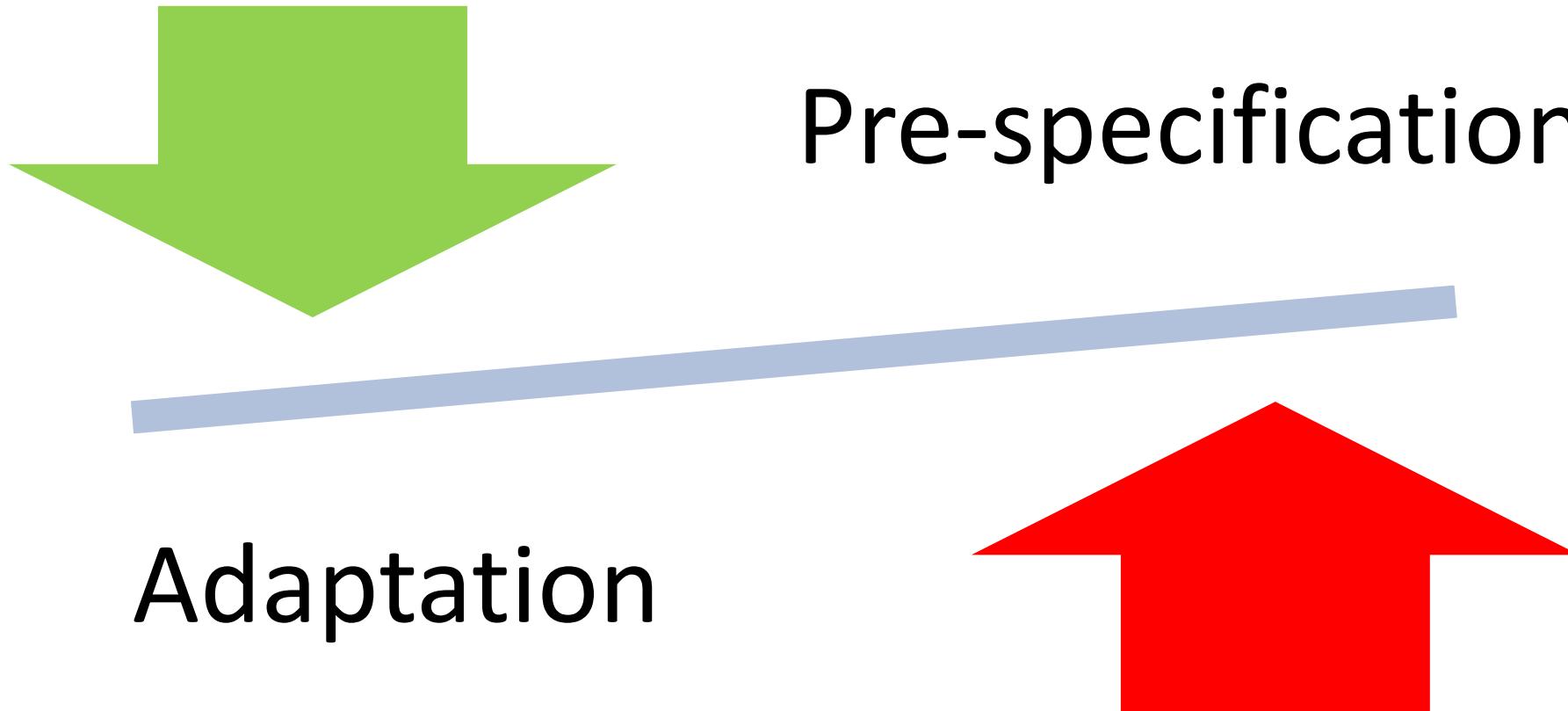
# Development of A Pediatric Knowledge Base



# Pediatric Drug Development



# Timing and Pediatric Drug Development



# Goals of ADEPT 7

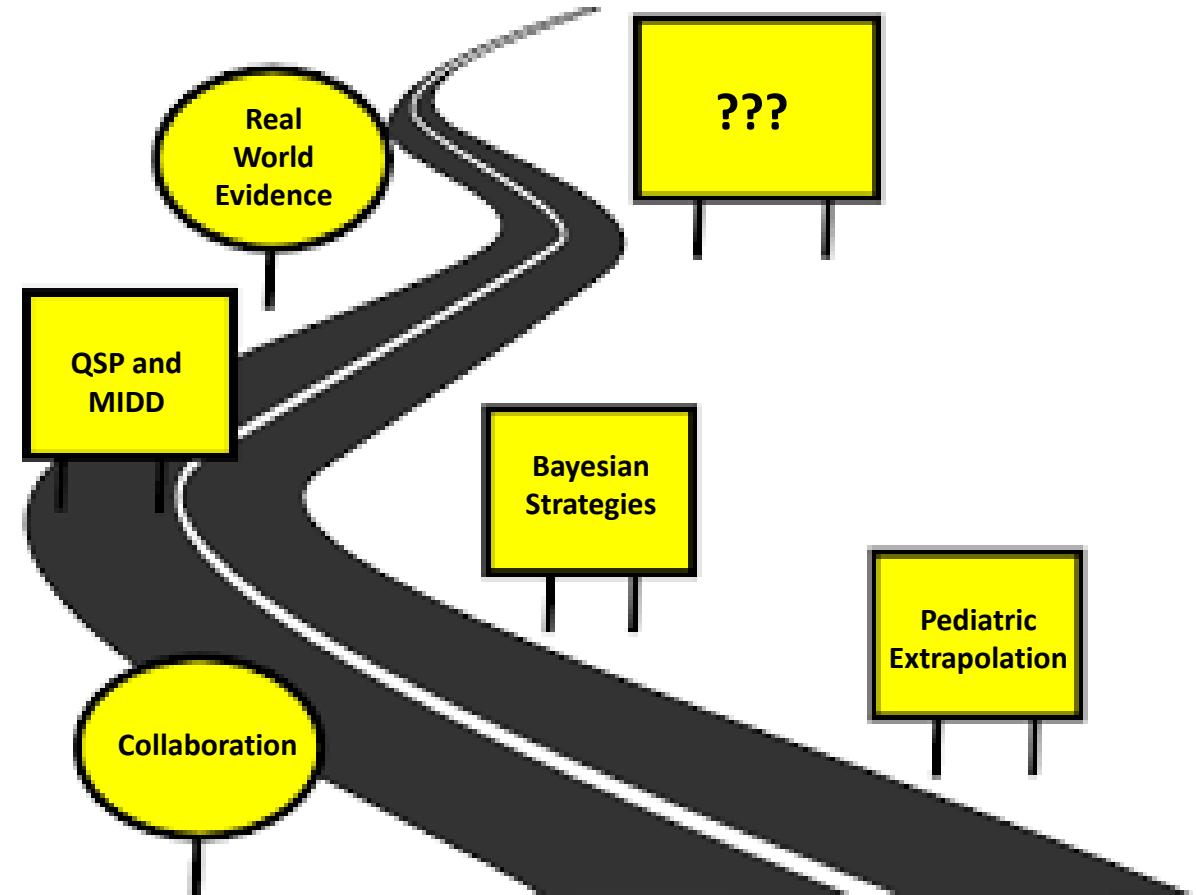
- Provide a platform for scientific exchange among the FDA, other global health authorities, patient advocates, and drug developers
- Discuss application and challenges of each topic in pediatric drug development and explore how challenges in the designs can be overcome
  - Use of Bridging Biomarkers in Pediatric Extrapolation
  - Use of Bayesian techniques in Pediatric Studies

# Topics to be Discussed

- Use of Bridging Biomarkers in Pediatric Extrapolation
  - How can they be used in pediatric product development?
  - What criteria should be required to support their use?
  - What data should be collected to support their use?
- Use of Bayesian techniques in Pediatric Studies
  - FDA Perspective
  - EMA Perspective
  - Industry Perspective

# Advancing Development of Pediatric Therapeutics

- Adherence to regulatory and ethical requirements for approval of therapeutics in children
- How do we meet the needs of pediatric patients as efficiently as possible?
- How do we maximize the use of all relevant data?
- How do we maximize the confidence in our decision-making?



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