

Global Network Collaborations

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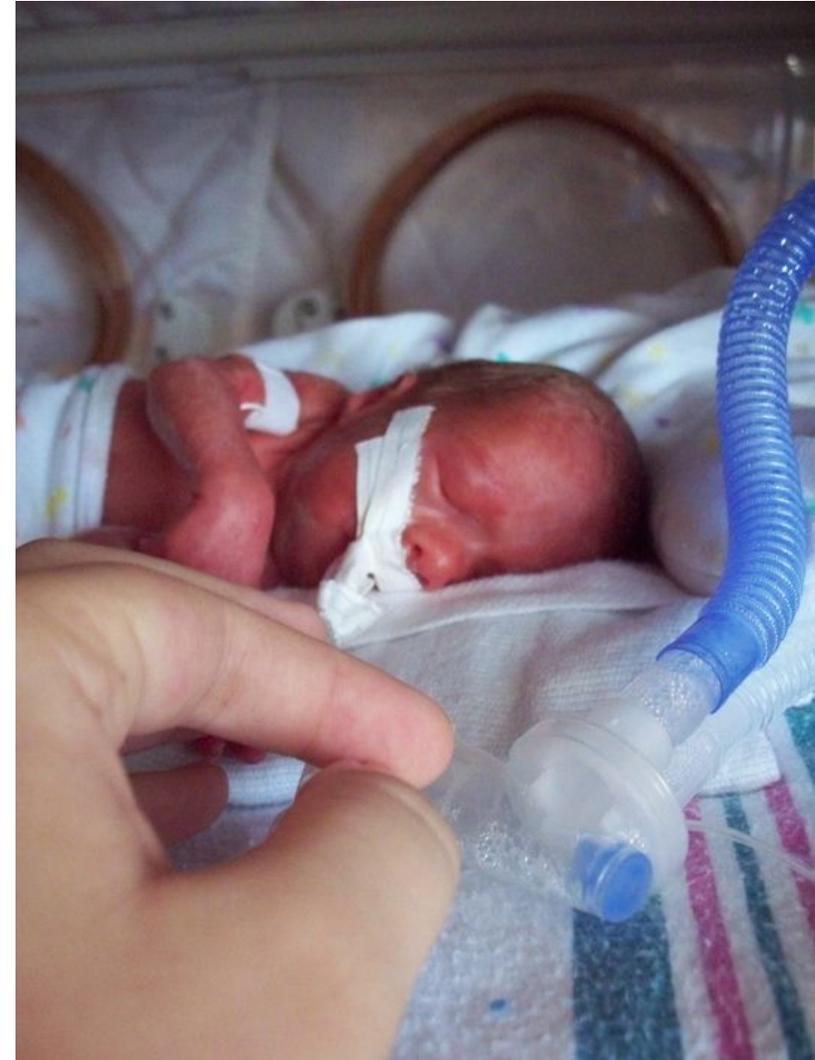
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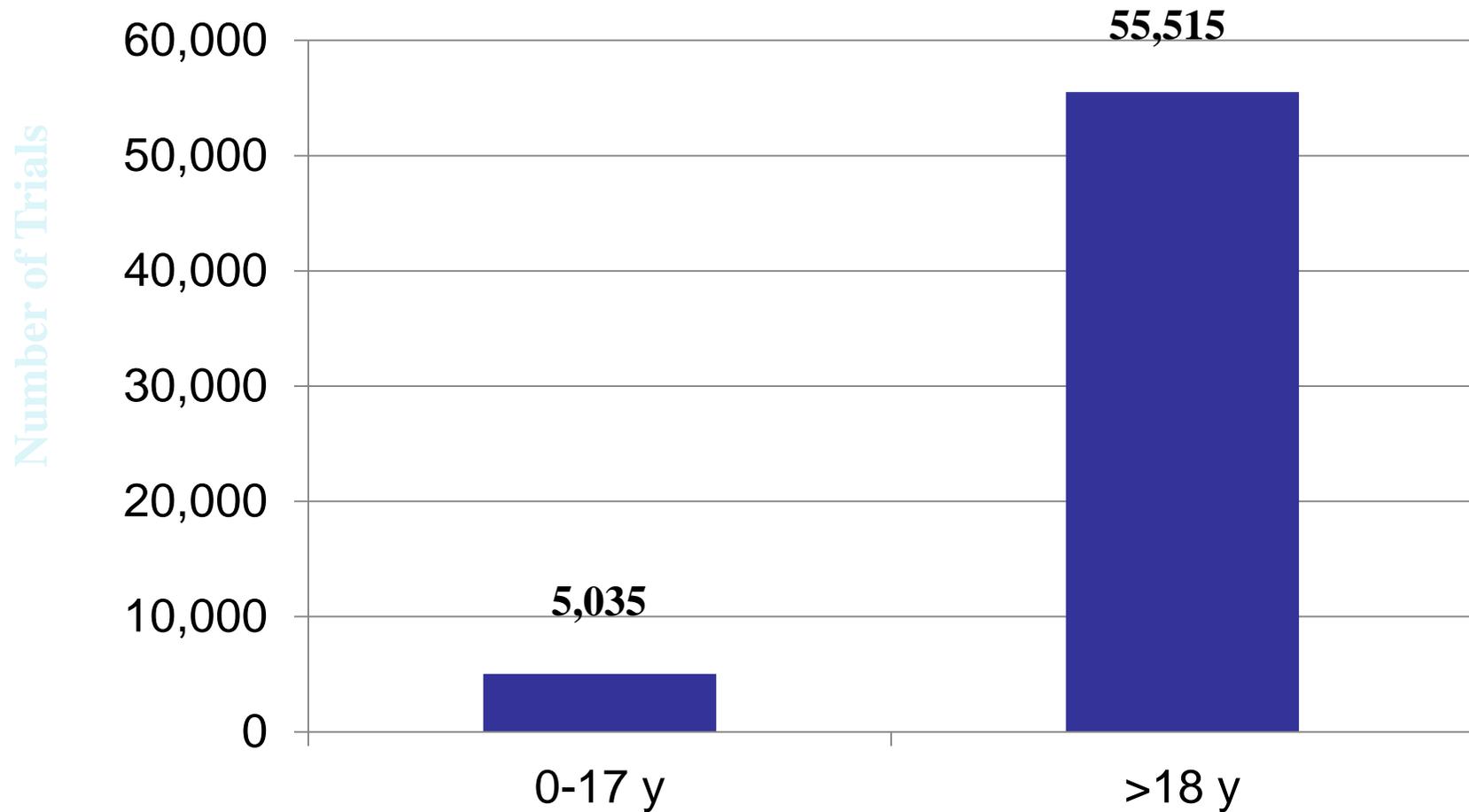


No financial information to disclose

No conflict of interest exists

ClinicalTrials.gov

Children - 25% of the population



Developing Drugs for Infants & Children: National and Global Efforts

- **Studies require attention to many details**
- **Designing pediatric studies is “Team Science” - pediatricians, pharmacologists, statisticians, bioethicists, regulators, support staff, foundations, *families***
- **Better communication/collaboration among FDA, NIH, Industry, CROs, and Academia**
- **Network initiatives most promising**

Optimizing Outcomes for Infants and Children

- **Studies using single CDA, contracts, IRB**
- **Strategies to enhance patient recruitment**
- **Early identification of consultants in multiple sub-specialties for protocol development**
- **Setting up multispecialty, multinational, pediatric clinical trial enterprises**
- **Will you participate, especially with industry sponsored trials?**
- **We must change the traditional academic model**

US Initiatives to Facilitate Pediatric Drug Development

- **FDA: Major legislative initiatives (1997) to require and incentivize industry to develop pediatric products while enhancing pediatric resources & programs at FDA**
- **NIH: Monthly conferences with FDA addressing many scientific and product specific issues**
- **NIH: Pediatric Trials Network (NICHD) and sponsored clinical trial networks (CTSA)**
- **NIH & FDA legislative mandate: “Docket process” for older and sponsor refused products**
- **Orphan Drugs/Rare Diseases: significant incentives**

Orphan Product Designation and Its Benefits

- Orphan drugs are intended for the safe and effective treatment of diseases/disorders that affect $\leq 200,000$ people in the U.S. or affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a specific treatment
- How to apply for orphan drug designation and tips for submission:
 - <http://www.fda.gov/forindustry/developingproductsforrareconditions/howtoapplyfororphanproductdesignation/ucm365086.htm>
 - Contact for questions: Jeff Fritsch R.Ph. (Jeff.Fritsch@fda.hhs.gov); Tel:301-796-8682)
- Benefits if application granted:
 - seven years market exclusivity for a specific indication following the approval of the product by FDA
 - qualifies the sponsor of the product for the tax credits
 - NDA is not subject to a prescription drug user fee unless the application includes an indication for other than a rare disease or condition

FDA Orphan Product Grant Program

- **Goals:** to get orphan products to market or add or change label
- **Scope of the orphan product grant program**
 - Funds for human clinical trials: test the safety and efficacy of promising **new** or **repurposed** drugs, biologics, devices, and medical foods for rare diseases and conditions
 - Up to 4 years
 - Each year, there are ~80 - 90 ongoing grant-funded projects
- **Contact information:**

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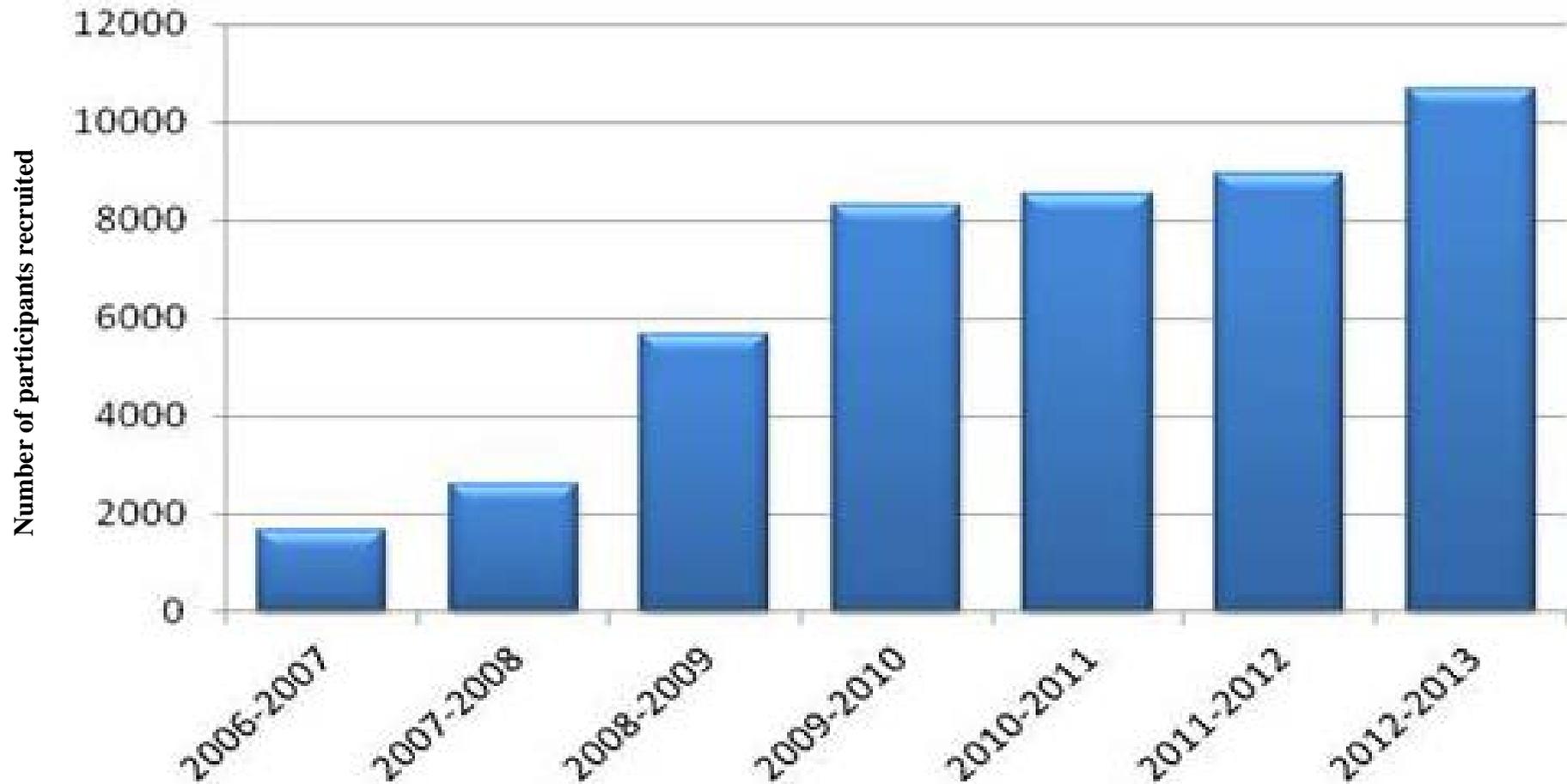
Pediatric Product Development: Global

- **Since 2007, Europe has had similar Pediatric legislation with respect to requirements and incentives**
- **Europe mandates an “earlier” determination of appropriate studies for children**
- **Countries can still generate pediatric data for just the national authorization OR as part of the EMA**
- **Medicine for Children Research Network (MCRN) – UK is an example of a program that has “turned things around”**
- **Japan is an active pediatric participant; Canada and Australia are part of FDA Cluster; Latin America now conducting almost 20% of US sponsored clinical trials**
- **Chinese regulators visiting FDA and showing interest**

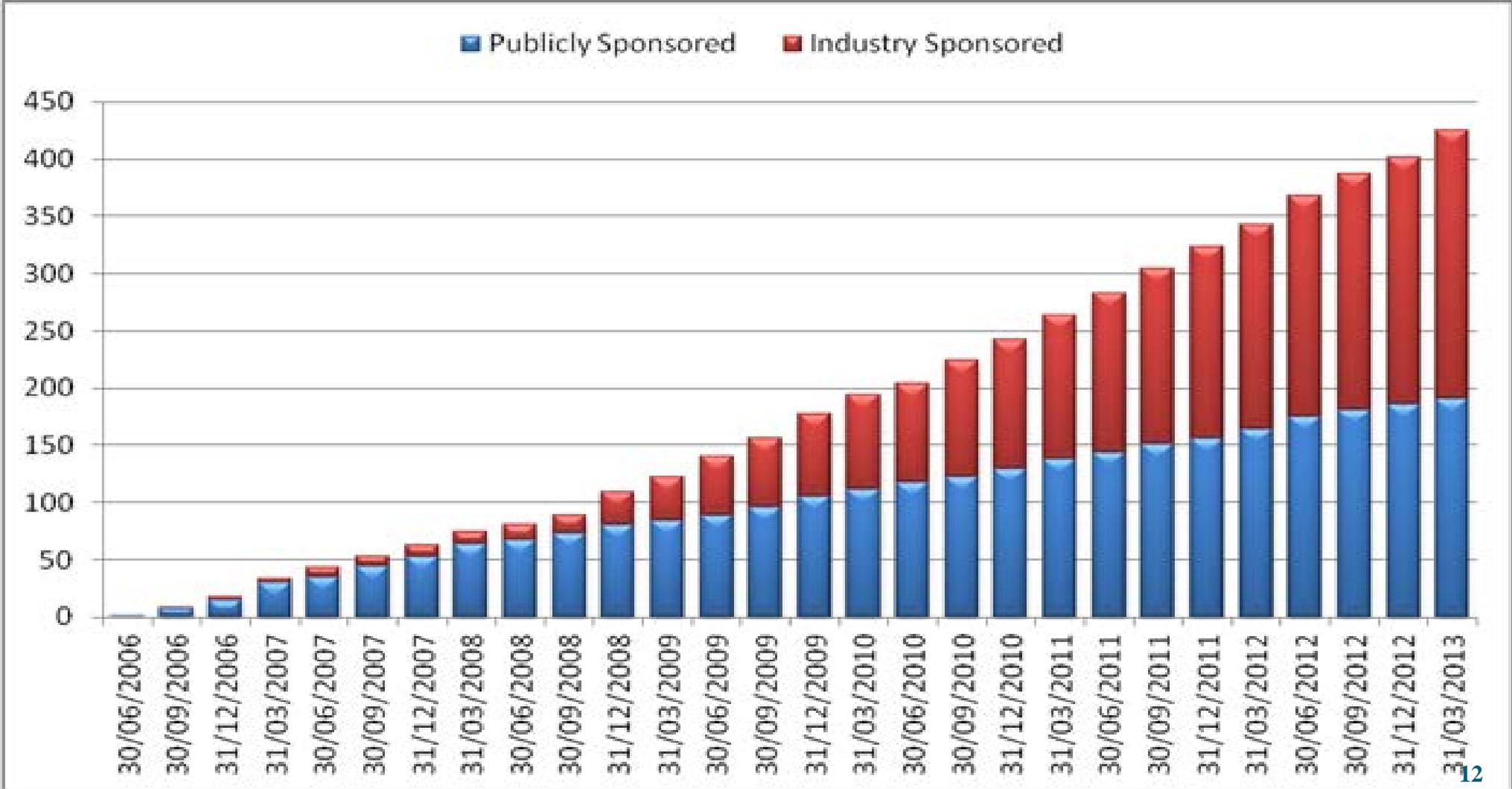
MCRN Clinical Trials Group

- **Build infrastructure to conduct pediatric clinical trials**
- **Streamline clinical trial processes**
- **Key opinion leader advisory groups**
 - **Identify research priorities within specialty areas**
 - **Propose and develop trial ideas and designs**
 - **Work with investigators to develop study ideas leading to successful funding applications**
 - **Provide methodological advice to industry and other investigators (protocols, PIPs etc)**
 - **Site selection and performance metrics**

Recruitment to MCRN Studies



Growth of MCRN Portfolio



WORKING IN PARTNERSHIP WITH YOUNG PEOPLE

MCRN Children & Young Person's Groups



Global Research in Pediatrics (GRiP)

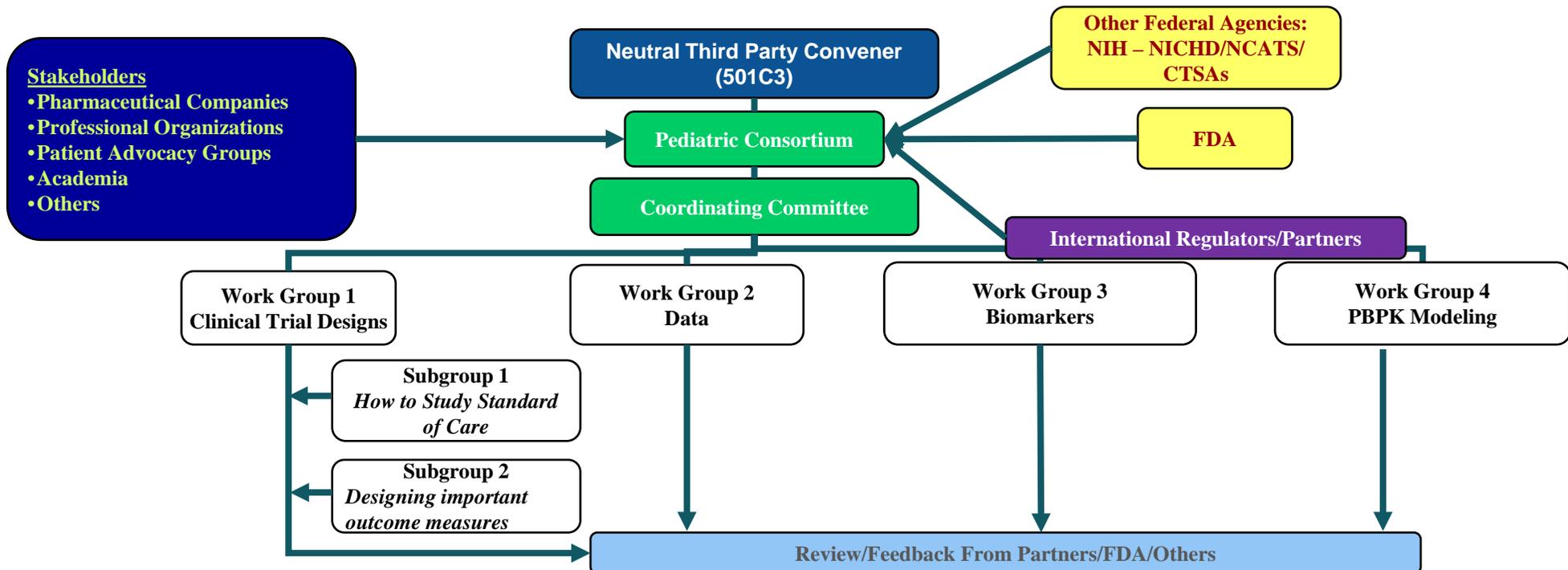
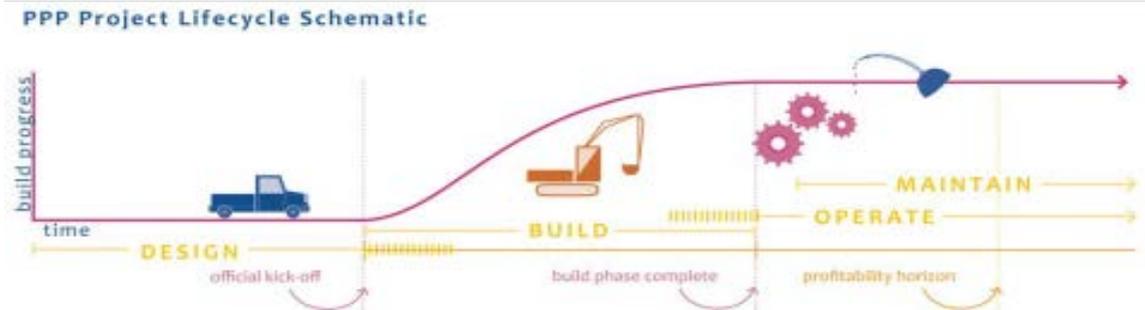
- **EU funded initiative – facilitate development of safe and effective medicines for children**
- **Establish best practices in pediatric research**
- **Set up adequate infrastructure to perform clinical trials**
- **Development of comprehensive training programs (pharmacology, clinical research)**
- **Focus on regulatory science**
- **Collaborations with EU, US, Canada, Japan**

Global Initiatives to Facilitate Pediatric Drug Development

- **Multisite clinical trials with sites from US, EU, and Canada conducting the same protocol**
- **Enabled by better communication among regulatory agencies**
- **Ability to come to consensus on study design and relevant outcome measures**
- **Will permit approval in multiple countries simultaneously and save years off the development process**

Pediatric Consortium Concepts

- Leverage the experience of non-profit neutral third party conveners who have experience in trial design & drug development tools
- FDA has experience with public-private partnerships - clinical trial design, disease models, biomarkers, outcome assessments, tools for qualification, & data standards
- A number of external experts are interested in collaborating (e.g. NIH, industry, academia)





Advancing Maternal - Child Health

Sustainable
Infrastructure

Cooperative
Networks

Knowledgeable
Workforce

Efficient
Regulatory Processes