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Bio

Why Pediatric Clinical Trial Networks?

Current Challenges to Run Global Paediatric Interventional Clinical Trials

CHILD

Child Health Innovation Leadership Department

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June 5, 2019

As a Society We Have Work To Do…

Over 50%

of medicines used to treat children have not been studied in children

Over 90%

of them have not been studied in infants

Different Doses, Different Formulations

Small Populations and Sub-Populations

At least Five Pediatric Sub-Populations

Preterm Newborn Infants	Term Newborn Infants	Infants and Toddlers	Children	Adolescents
Pre-term	*0–28 days*	*29 days to 23 months*	*2 Years to 11 Years*	*12 Years to 18 Years*

- Heterogenous population
- Low incidence of diseases
- For each sub-population, separate clinical studies are often required
- Eligibility criteria further narrow the pool eligible to be enrolled in a study

Adult vs. Pediatric Trial with the Same Anti-hypertensive

Adult

- N = 220 subjects
- Countries: One (USA)
- No. Sites: 9
- Study time: 5 months
- 24 subjects per site

Pediatrics
(6-16 y/o)

- N = 253 subjects
- Countries: Three
- No. Sites: 70
- Study time: 1 year
- 3 – 5 subjects per site

Thank you to Dr. Ron Portman for providing this example

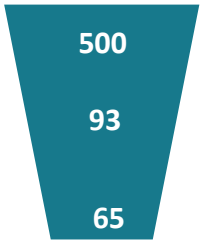
Opportunities for Efficiency

Efficiency lowers R&D investment and increases likelihood of success

Real Experience

1

Site Selection & Throughput



Approached

Selected

Enrolled Patients

2-5 Patients Per Site over 1-2 Years

2

Aligning CDA, Contracts and have a single ICF

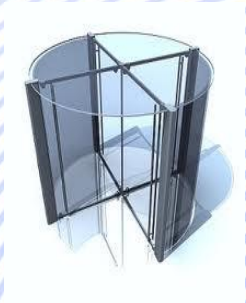
3

Minimize Protocol Amendments

9 amendments slowed enrollment

4

Staffing Consistency

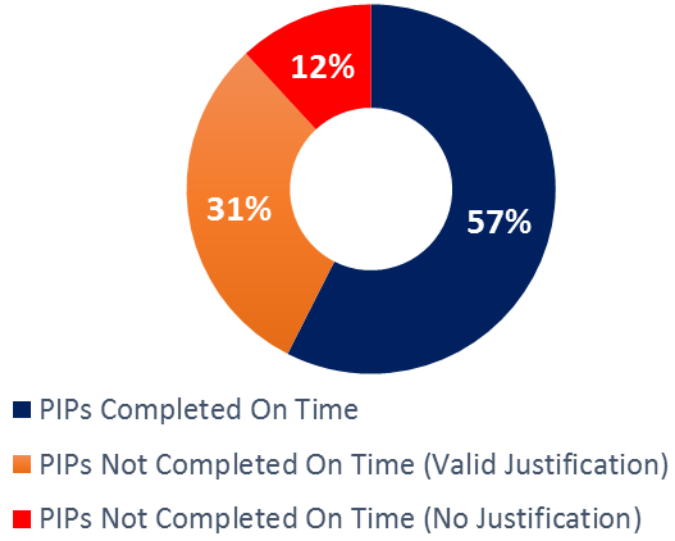


Paediatric Trials – Still Struggling

Europe

Status of PIP Completion
Snapshot as of June 2017
N = 261 PIPs

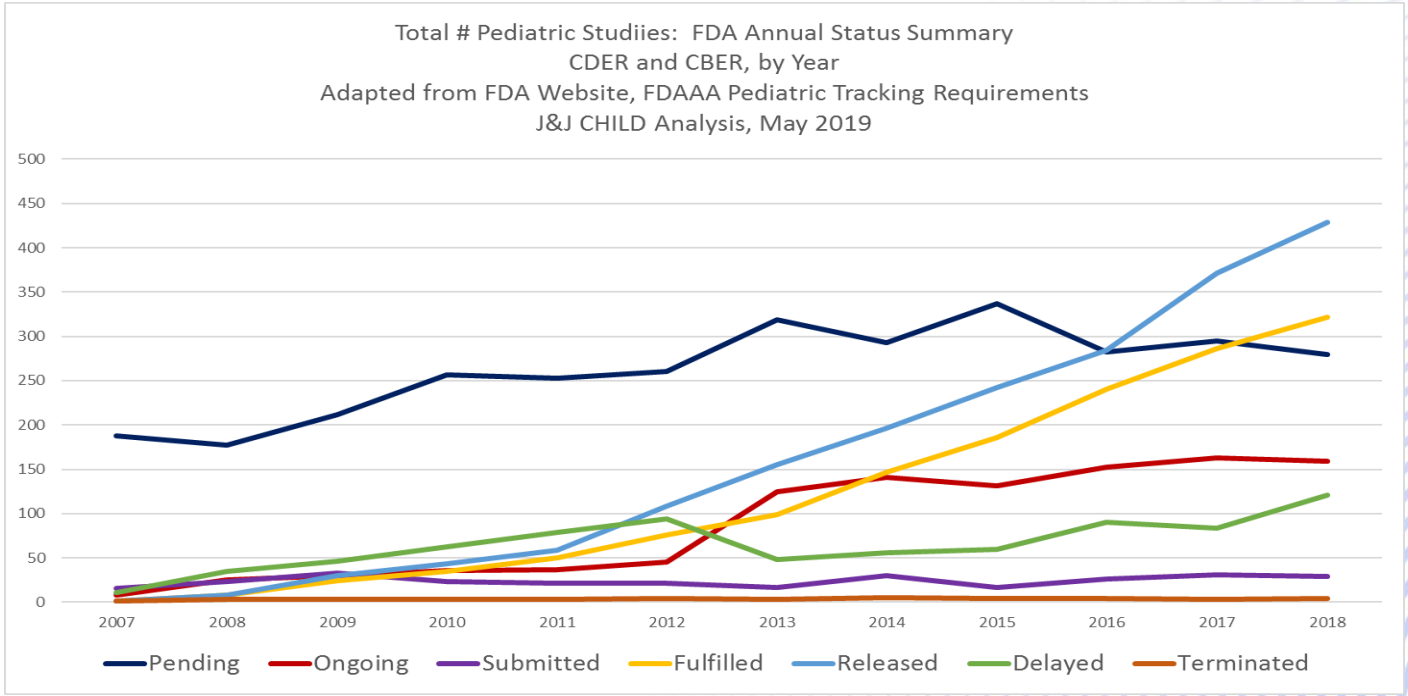
Source: Adapted from EMA Annual Report to the European Commission dated April 2018, J&J CHILD Analysis, May 2019



Pediatric Trials – Still Struggling

Annual Snap Shot at Year End
Last 5 Years ~60% Have Not Yet Enrolled Patients or Have Been Released from Obligations

United States



Potential Solutions?

Pediatric Clinical Trials Networks

c4c *

IACT *

Others

PAVING THE FUTURE FOR THE TREATMENT OF PAEDIATRIC DISEASES THROUGH A PAN-EUROPEAN CLINICAL TRIALS NETWORK

c4c

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 777389.
The Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.

Vision

Better medicines for babies, children and young people through a pan-European clinical trial network

MISSION

c4c will use a coordinated approach to deliver high quality “regulatory grade” clinical trials in:

- Multiple countries
- Multiple sites
- All paediatric age groups

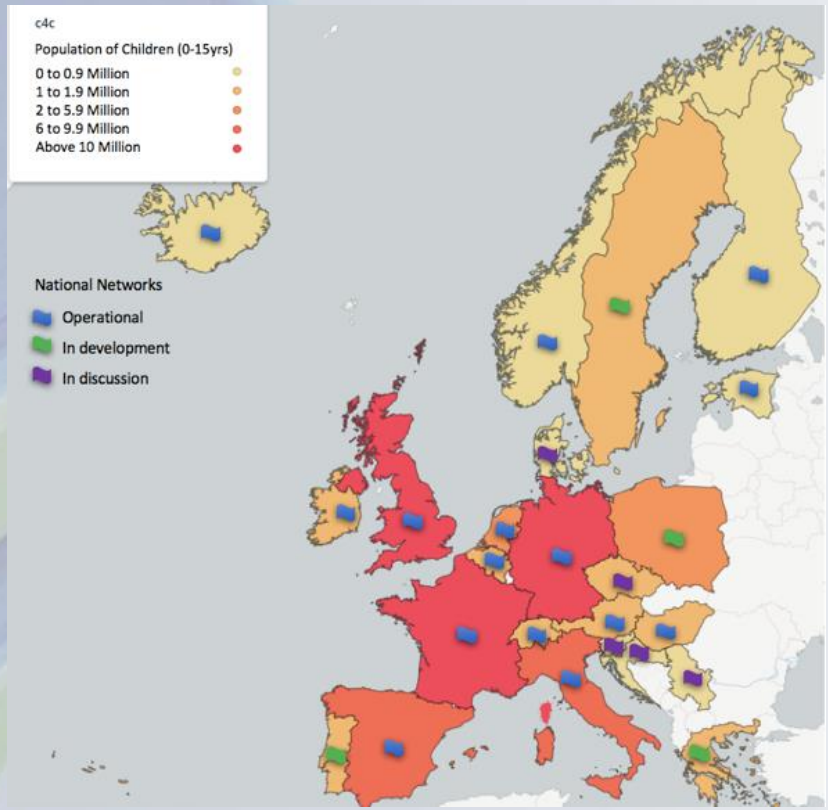
by supporting:

- Trial implementation using resources shared between studies
- Trial design through a combination of information about natural history, feasibility, appropriate innovation, and expert opinion
- Education and awareness within and beyond the network

Key Objectives

- More efficient trial implementation through the set-up of **national hubs** and qualified sites
- Input in clinical trial design and implementation from **pilot expert advisory groups** and other fora
- **Educational programme** for health professionals and **awareness raising campaigns** for the general public
- Identification of **Data standards** and performance metrics
- Business cases for **sustainability** beyond IMI funding

The c4c consortium members



- 10 EFPIA companies
- 18 paediatric national networks
- 2 large patient advocacy groups
- 8 EU multinational specialty networks
- 3 global research networks
- 2 large children's hospitals

Institute for Advanced Clinical Trials for Children

I-ACT INSTITUTE FOR ADVANCED CLINICAL TRIALS FOR CHILDREN

Core Elements of I-ACT's Approach

I-ACT for Children is an independent 501(c)3 public-private collaboration designed to advance innovative medicines and device development and labeling to improve child health.

Pediatric Product Development

Skills and experience for regulated pediatric product development, labeling and post-marketing for innovative medicines and devices

Innovation

Clinical development strategy and trial design

Efficiency

Operational efficiency with high quality

The Current U.S. Site Network

44 US sites as of March 2019; 2019 goal of 60 sites

- Pre-competitive projects
- Advice & guidance on proprietary projects
- Facilitation of clinical trials, feasibility
- Trial conduct, enrollment etc.

- Innovative medicines and devices
- Regulatory-quality clinical trials
- Therapeutic-area agnostic
- Partnered with specialty & international networks
- Participate in I-ACT for Children metrics & QI program
- Utilize central IRB
- Contract agreements in place
- Dedicated medical & operational points of contact
- Peer-to-peer engagement
- Mentoring program
- Communications & troubleshooting

Key Global Collaborators - GPCTN

Duke Clinical Research Institute — FROM THOUGHT LEADERSHIP TO CLINICAL PRACTICE

conect 4children — COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

MICYRN — better health for mothers and children

Japan Clinical Trials Network

Example Advocacy Relationships

ICAN — International Children's Advisory Network

Parent Project Muscular Dystrophy

PPA — Preemie Parent Alliance

Example Research Alliance Organizations

PEDSnet — A Pediatric Learning Health System

Nationwide Children's Hospital

CRITICAL PATH INSTITUTE

National Capital Consortium for Pediatric Device Innovation

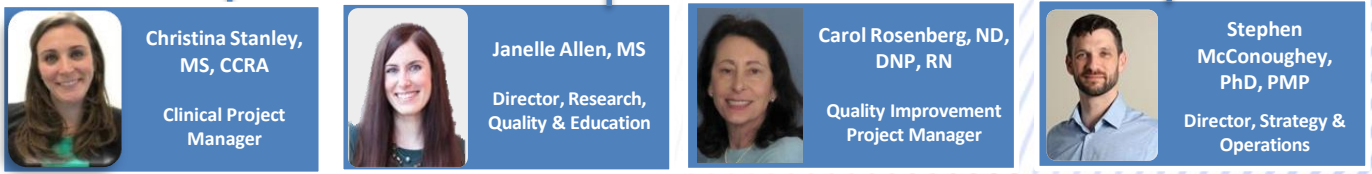
Cincinnati Children's

James M. Anderson Center for Health Systems Excellence

Tufts CTSI — Tufts Clinical and Translational Science Institute

Pediatric Specialty Networks

I-ACT for Children: Key Staff



Thank you