

Current Network Structure for Pediatrics

Country, state, regional,
networks

Hospital Networks

Academic networks

Sub-specialty
networks



NIH networks

Foundation networks

Trial recruitment networks

Disease specific networks



Networks for Pediatric Drug Development

- European network for pediatric research (enpr-EMA):
 - e.g., PRINTO, PENTA, ITCC, BFM, MCRN, European Research Network in Diabetes and Endocrinology
- US:
 - NIH related networks
 - Phase 1: Pediatric Trials Network (off-patent) (former PPRU)
 - Phase 3: CTSA, COG, PHN, neonatal
 - Association/Foundation networks
 - CFF, NAPRTCS, AAP PROS, Rare disease
 - Group or individual sites:
 - many, fragmented, disorganized, not ready for clinical trials, no incentive for faculty participation
 - Preferred site program: multiple academic sites and children's hospitals in US and EU
- Global:
 - PAIDION (newly forming pediatric research organization)
 - Japan, Canada, Brazil, Argentina, Mexico, Australia



BIO and TransCelerate

- *“TransCelerate BioPharma will develop shared industry research and development solutions to simplify and accelerate the delivery of innovative products to patients. Our non-profit, procompetitive model will be based on a results-oriented approach, emphasizing increased quality in clinical studies and improved patient safety, enabled by broad participation and collaboration across the global research and development community.”*
- *Made up of 16 BioPharma companies: projects: risk based monitoring, shared site qualification and monitoring, common investigative site portal, clinical data standards – efficacy, comparative drugs for clinical trials.*
- BIO is committed to improving efficiency of pediatric clinical research
- BIO has partnered with ViS Research and is leveraging their analytics platform to better understand the global pediatric research infrastructure
- Senior Leadership from TransCelerate has publicly expressed interest in working together to develop and implement a pediatric clinical trial network
- BIO is in the very early stages of identifying additional partners and developing timelines for the network

<http://transceleratebiopharmainc.com>, www.bio.org, www.ViSresearch.org



Key Factors for Operational Success

- **I**nnovation in study design
 - Innovative study designs: extrapolation, pharmacometrics, multi-company studies, adaptive designs, quantitative extrapolation
 - Registries
 - Repurposing of existing samples for PK
 - New technologies for identifying patients and physicians globally
- **P**rotocol(s)
 - Designed with experts to answer valid scientific questions with feasibility and expedience
 - Approved by regulatory agencies (global)
 - Approved by IRB
 - Developed in conjunction with operational network or CRO
- **R**ecruitment
 - Based on disease frequency
 - Countries, sites, investigators, patient population
- **E**nrollment and Retention
 - Study team



enpr-EMA Processes

Internal factors

- Not transparent to industry
- Structure in itself is an achievement
- Added new networks
- Standard PIPs
- Patient group involvement
- Interaction with EMA re: feasibility

Interactions with industry

- Communications are difficult and process for consultation not clear for individual sites nor central administration
- Is there a single process or policy or different for each network?
- Role of individual academicians and sites within the networks
- Role of network in consultation with EMA?
- Confidentiality issues: process and time
- Timely consultation
- Conflict of interest: EMA vs Industry
- Industry is not really a part of enpr-EMA



Enpr-EMA Success

- PRINTO, PENTA, ITCC, BFM, MCRN, European Research Network in Diabetes and Endocrinology
- Printo (PRCSG) major role in developing and evaluating new therapies for JIA in children
- PENTA: HIV
- ITCC and BFM for oncology studies; BFM for anticoagulants (COG)
- MCRN multiple successes
- Diabetes group stimulated recent EMA meeting on T2DM in Feb 2013 that will hopefully allow a redesign of studies to allow for successful completion of these programs where failure was almost certain
 - Identification of poor enrollment; large numbers of studies and patients required, need for innovative study design

