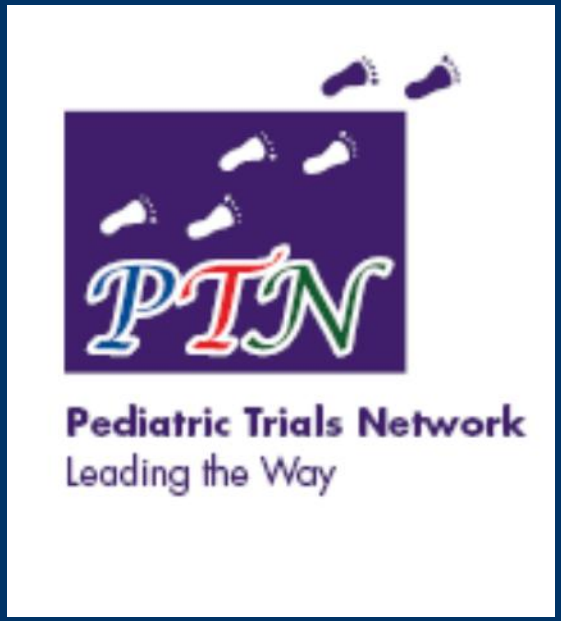
Pediatric Trials Network



Duke Clinical Research Institute

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www.dcri.org/about-us/conflict-of-interest

Pediatric Drug Development

- 1998: essentially no trials
- Mandate (Pediatric Research Equity Act)
- Incentive (Exclusivity)
- Virtually no studies in young infants
- Off Patent (Best Pharmaceuticals Children Act)
  - Authorization by Congress
  - NICHD sponsored trials
  - 2002-2010
  - 6 molecules, one trial enrolled on-time

What Is The Pediatric Trials Network PTN?

- Sponsored by the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)

- The primary objective of the Pediatric Trials Network:

Create an infrastructure for investigators to conduct trials that improve pediatric labeling and child health.

- PTN is studying product formulation, drug dose, efficacy, safety, and device validation
- Evidence of success will be completed trials that improve dosing, safety information, labeling, and ultimately child health

Pediatric Trials Network (PTN) 2011

Project	Date Signed	Protocol Status	Open to enrollment
TO1 Administration	Oct 2010	NA	NA
TO2 - Hypertension protocol	Oct 2010	Protocol Complete	NA
TO3- Metronidazole	Dec 2010	Protocol Complete	Complete
TO4 - Hydroxyurea	May 2011	Protocol Complete	Interim analysis on time
TO5 Acyclovir	Jun 2011	Protocol Complete	Interim analysis, on time
TO6 Opportunistic (POPS)	Aug 2011	Protocol Complete	Yes, on time
TO7 -Lisinopril PK	Aug 2011	Protocol Complete	Yes, on time
TO8 -Tape	Sep 2011	Protocol Complete	Complete
TO9 -Midazolam	Sep 2011	Protocol in draft	NA
TO10 Ampicillin	Sep 2011	Protocol Complete	NA
TO11 -Obesity	Sep 2011	Protocol Complete	NA

Lessons Learned Main Contract Timelines

- Meropenem RFP release to signature 24 months
- IND 31 months
- First patient 34 months
- Last infant 48 months
- Clinical Study Report 60 months from RFP release

- Pediatric Trials Network RFP 3/2010, signature 6 months
- IND 7 months
- First patient 9 months
- Last patient 18 months
- Clinical study report 22 months from RFP release

Innovations and Track Record That Made it Go

- DCRI operations team—job vs. mission
  - Contracting—risk to NIH and to investigators
  - POPS
  - IRB
- Meropenem—give it away to keep it
- Trial leadership and PI selection
- Per patient cost—between and within trial
- Success in first trial—support of NICHD
- Success in 2011—support of the members
- Only decisions that impact timelines and budget

Protocol: POPS Pediatric Opportunistic PK Study

- Protocol Title: Pharmacokinetics of Understudied Drugs Administered to Children per Standard of Care
- Objectives:
  - Evaluate the PK of understudied drugs currently being administered to children.
- Study Population: 500 children (birth-20 years) who are receiving understudied drugs of interest per standard of care as prescribed by their treating caregiver
- Study Duration: each child will participate in the study for up to 90 days per drug; study conduct for 3 years
- Number of Sites: 45
- First Patient Enrolled: November, 2011

PTN and POPS Continued

- 15 therapeutics bundled into one protocol
- Samples stored locally and sent in batch
- Flexibility to add molecules
- Provide preliminary and supportive data for subsequent trials
  - Compare to epi-data
  - Metronidazole example
- Provide a testing ground for sites—enrollment
- Facilitate contracts and infrastructure—enrollment in between more traditional trials

Comparison Legacy Trials Pediatric Trials Network

- Legacy 10 years
  - website
  - 6 molecules
  - 1 trial completed on time
- Pediatric Trials Network
  - 30 molecules
  - All trials on time and on budget to date
  - 16 trials over 7 years requested, will have started 14 trials in 2 years
  - 2 CSR
  - Website www.pediatrictrials.org

Applications infectious disease trials

- Success in ID trials already
- POPS
  - Site selection and reduce start up time
  - Post-marketing safety
  - Feasibility
- Interacting with industry
- Pharmaco-epi
- Piggyback of diagnostics


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