

Partnering to Optimize & Accelerate Pediatric Clinical Trials



Update on Collaborative Networks-Enpra-EMA Workshop

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1 www.iactc.org

Mission and Vision



Mission: To serve as a neutral and independent organization on behalf of children everywhere, bringing a dedicated voice to the advancement of new medicines and devices needed now and in the future. Our work is to engage public and private stakeholders through research and education to ensure that healthcare for children is continually improved by enhancing awareness, quality and support for pediatric clinical trials.

Vision: We act because every child with a medical need deserves the best chance possible. That chance depends on a commitment to innovation, quality and urgency in advancing medical therapies specifically for children.

Our Focus and Scope

Strategy & Planning

Innovation and efficiency



- Innovative trial design
- Site feasibility assessment
- Independent assessment of programs/strategy, including PIPs, PSPs, protocols
- Protocol optimization
- Use of real-world data

Infrastructure & Trial Execution

Access and global reach



- Geographically diverse centers of excellence
- Pre-qualified trial-ready sites
- Patient recruitment strategies/materials
- Efficiency metrics, troubleshooting, training

Tools, Capabilities, Best Practices

Consistency and competency



- Standard processes and practices
- Site optimization
- Centralized ethics review
- Pediatric-based GCP and other education

Thought Leadership

Experience and proficiency



- Consult on all aspects of pediatric trials, including protocols and processes
- Deep understanding of the regulatory landscape

Priority Areas

- Common scientific approach between US and other jurisdictions
- Making product development more patient-centered
- Developing a rapidly deployable clinical research planning teams
- Efficient and sustainable infrastructure
- Robust interoperable data – real world evidence
- Leveraging innovation
 - Pediatric Extrapolation
 - Innovative Trial Designs and Model-Informed Drug Development
 - Mechanism-of-Action Based Development
 - Strengthening the Evidence Base for Medicines and Devices Used in Neonates
 - Developmentally appropriate Endpoints/Biomarkers
 - Better Feasibility and Trial Concepts
 - Age-Appropriate Formulations
- Education and workforce development
- Continuous learning and quality improvement
- Developing shared tools

Site Support and Engagement

- Our growing site network is built to bring state-of-the-art to pediatric trials
 - Each site has a Clinical and Operational Lead dedicated to site metrics and outcomes
 - Sites actively engage in best practice and educational committee
 - Will be trained in pediatric-specific GCPs (fall-winter 2018)
 - Will have access to a central IRB (estimated completion 2018)
 - Listserv planned for coordinator staff to share best practices, address challenges
- At the same time, we provide:
 - At the same time, we provide:
 - Peer-to-peer engagement to encourage investigator/patient retention, energize recruitment, accelerate study milestones
 - CRO training on pediatric research & site management
 - Site problem-solving with I-ACT's operational team
 - Patient engagement & education tools
 - Recruitment support for minority populations
 - Representation on the Best Practices, Education and Tools Committee

Current U.S. Network



1. Seattle Children's Hospital		
2. Rady Children's Hospital		
3. University of Utah Children's Hospital		
4. Children's Hospital Colorado		
5. Children's Mercy Hospital		
6. University of Minnesota Masonic Children's Hosp.		
7. Arkansas Children's Hospital		
8. St. Louis Children's Hospital	16. Children's Hospital of Philadelphia	24. Dell Children's Medical Center
9. Ann & Robert H. Lurie Children's Hospital of Chicago	17. Columbia University-Morgan Stanley Children's Hosp.	Lucile Packard Children's Hospital (Stanford University)
10. Riley Hospital for Children	18. Connecticut Children's Medical Center	25. Driscoll Children's Hospital
11. Cincinnati Children's Hospital Medical Center	19. Floating Hospital for Children at Tufts Medical Ctr	26. John's Hopkins Children's Center
12. Children's Health Care of Atlanta at Egleston Hosp.	20. Boston Children's Hospital	27. Texas Children's Hospital
13. Nationwide Children's Hospital	21. Arnold Palmer Children's Hospital	Tulane Lakeside Hospital for Women & Children
14. Nemours Children's Health System	22. Children's Hospital of Orange County	28. Cook Children's Medical Center
15. Children's National Medical Center	23. Cook Children's Medical Center	29. Children's

Our Global Vision

- 29 US sites as of May 2018; EOY goal of 40+ sites, including 5 ex-US
- 100+ global sites by 2019-2020
- Relationships with C4C, Canada, Japan
- Partnerships with specialty networks



Rapidly Deployable Trial Planning- Clinical Study Groups (CSGs)

- Goal: Build a group of rapidly accessible experts to address both proprietary and non-proprietary needs.
- Clinical experts aligned by subspecialty and/or discipline (ethics, regulatory affairs) and patient-parent advocates
- Areas of collaboration
 - Consult on proprietary project teams
 - Public forum and master trial synthesis
 - Advocacy for clinical trial timely initiatives in pediatric patients
 - Educational efforts

I-ACT Work in Progress-Proprietary

- Providing counsel on the design of phase 2 and 3 trials for a cardiovascular indication, including elements such as sample size, duration and event measurement
- Working to optimize Phase 2 study execution for a neuroscience trial, including trial site identification and assessment, patient recruitment and retention strategy and ongoing trial site engagement
- Launching a project designed to enhance trial-site recruitment for a pediatric study for a the metabolic-endocrine indication, including using observational and real-world data to identify and assess the available patient population.

I-ACT Work in Progress-Non-Proprietary

- Partnership with Critical Path Institute and a parenting group to develop a master protocol for Duchene Muscular Dystrophy
 - Multi-arm platform trial with shared control group
 - Initial FDA meeting 5-2018
 - Public workshop-winter-2018
- Collaboration with the National Pediatric Device National Capital Consortium for Pediatric Device Innovation (NCCPDI)
 - Public workshop meeting on pediatric device development-Fall 2018
 - White Paper/Guidance for Industry and IRBs
- Ongoing discussions with regarding formation of a consortia in PPH/PAH
- Establishing multiple sources for real world data access.

Key Committees-Scientific Advisory



John Bradley MD

Co-Chair

Professor of Pediatrics and Chief of the Division of Infectious Diseases

University of California - San Diego School of Medicine

Rady Children's Hospital

- Review of proprietary projects
- Portfolio advice and guidance
- Framework for review and prioritization of projects
 - Fit for *I-ACT for Children's* mission/vision and values
 - Potential for tangible child and public health impact
 - *I-ACT for Children's* added value
 - Portfolio management assessment and bandwidth
 - Level of *I-ACT for Children* engagement in strategy and planning
 - Membership engagement

Key Committees-External Affairs



John Davis, MD

Co-Chair

Professor of Pediatrics, Tufts University

Chair, Neonatal Advisory Committee, FDA

Director, Trial Innovation Center at Tufts CTSA Hub

Pamela Simpkins, MBA

Co-Chair

Senior Director, Strategy in the Child Health Innovation Leadership

Department, Office of the Chief Medical Officer, Johnson & Johnson

- Non-Proprietary scope
- Public-Private collaboration (industry, regulators and other agencies, investigators, parents, professional organizations, etc.)
 - Gaps and challenges in pediatric clinical trials
 - Scientific and educational priorities
 - Patient engagement approaches and methodologies
 - Collaboration opportunities with other pediatric networks and other stakeholders
 - Innovation in trials design and efficiency
 - Adoption of new technology and/or innovative scientific methods
 - Global interface and collaboration
 - New opportunities and challenges

External Advisory Committee

Co-Chairs:

Jonathan Davis, MD Floating Hospital for Children at Tufts Medical Center

Pam Simpkins, MBA Johnson & Johnson

Current Members (Non-governmental)

Susan Abdel-Rahman, PharmD U Missouri School of Medicine, Children's Mercy

AJ Allen, MD, PhD Eli Lilly

William Bentley, PhD University of Maryland

Barbara Bierer, MD Harvard Catalyst, Brigham & Women's Hospital

Florence Bourgeois, MD, MPH Boston Children's Hospital, Harvard

John Bradley, MD Rady Children's Hospital – San Diego

Martha Brumfield, PhD Critical Path Institute

Max Coppes, MD, PhD, MBA Renown Children's Hospital

Susan Ellenberg, PhD U of Pennsylvania Perelman School of Medicine

Kolaleh Eskandarian, PhD, MBA, PMP Children's National Medical Center

Chris Forrest, MD, PhD Children's Hospital of Philadelphia

Gigi Hirsch, MD Massachusetts Institute of Technology

Lynn Hudson, PhD Critical Path Institute

Jeffrey Hyams, MD Connecticut Children's Medical Center

Anne Junker, MD, FRCPC BC Children's Hospital, MICYRN

Peter Kim, MD, CM, PhD Children's National Health System

Samuel Maldonado, MD, MPH, FAAP Johnson & Johnson

Peter Margolis, MD, PhD Cincinnati Children's Hospital Medical Center

Hidefumi Nakamura, MD, PhD National Res Institute for Child Health & Development

Kathleen Neville, MD, MS Arkansas Children's Hospital

Kelly Parent, BS, CPHQ Beaumont Health System, University of Michigan

Lionel Phillips, MBA Inside Edge Consulting

Ron Portman, MD, FAAP, FASN, FASH Novartis

Klaus Romero, MD, MS, FCP Critical Path Institute

Harry Selker, MD, MSPH Tufts University School of Medicine

William Smoyer, MD Nationwide Children's Hospital, Ohio State University

Charles Thompson, MD, FAAP Pfizer

Mark Turner, MBChB (Hons), PhD University of Liverpool

John Van Den Anker, MD, PhD Children's National Health System

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