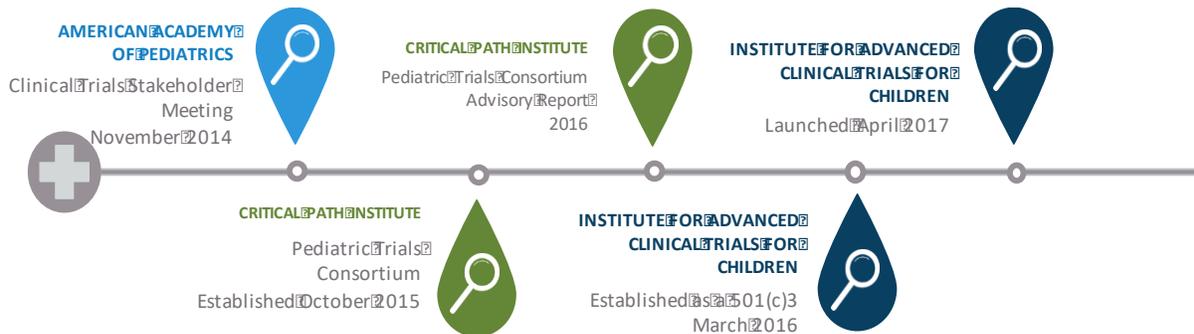




We act because every child with a medical need deserves the best chance possible. That chance depends on a commitment to innovation, quality and urgency in advancing medical therapies specifically for children.

THE CHALLENGE More than 50% of drugs used in children and 90% used in neonates are prescribed off-label, without adequate data. Innovative drug development in pediatrics remains slow and inefficient. It still takes 15 years to complete a pediatric drug development program; 9 years from approval of a drug in adults to a pediatric label. Overall, 40% of pediatric trials fail, 60% stall, and 4-6 avoidable amendments are needed per program. Only 1.5 pediatric patients are enrolled in trials per site per year and 30% of sites never enroll a single patient. The root causes are multifactorial, but key factors include misalignment of stakeholder interests, lack of an integrated and sustainable trials infrastructure, operational inefficiencies, and feasibility challenges.

WHO WE ARE We are an independent neutral 501(c)3 non-profit organization and believe that children of all ages deserve innovative medical therapies that are developed with the same level of urgency and commitment afforded adults. The Institute works with others to assure that studies are designed to generate sufficient data to allow safe and effective use of new medications and devices in pediatric populations. The history of *I-ACT for Children's* development is shown below:



HOW WE HAVE IMPACT A key factor in making this a reality is to optimize and accelerate biomedical innovation using child-centered clinical trial networks and collaboration with like-minded institutions, trial sponsors and other stakeholders. Together with parents, patients, investigators, foundations, regulators, other government agencies, biopharmaceutical sponsors and children's networks, we work to catalyze improvements in the quality and timely completion of pediatric studies to address the gap in evidence for best use of therapeutics in children.

 **Strategy and Planning** We provide independent, expert advice and guidance to sponsors and others, to help them develop pediatric plans and protocols – doing our best to get them “right the first time.” The core components of what we do include: facilitating the identification of unmet therapeutic needs; advancing scientific knowledge about best practices and strategies to close gaps in diseases and conditions that affect children; mobilizing stakeholders into action-planning to address important clinical trial design challenges.



Continued 



Capabilities, Tools and Best Practices We lead and participate in cross-sector teams that streamline and improve clinical trial processes to enhance the quality and timeliness of regulatory-quality data and reduce administrative burden. Education and training about standardized approaches and tools, particularly in those administrative areas that cause the most significant delays, are important outcomes of this work.



Infrastructure and Clinical Trial Execution We support and manage a network of pre-qualified trial-ready sites and collaborate with regional, national, international and disease-focused networks to ensure that we reach children across the world.



Leadership We serve as leaders to catalyze efforts that ensure early and continuous engagement of patients, caregivers, investigators, nurses, pharmacists and other research staff; create awareness and disseminate information and research regarding unique opportunities to address diseases and conditions that impact children; enhance the application of innovative trial designs and quantitative science research methods; improve clinical trial design and streamline trial conduct for all stakeholders.

Collectively, these activities build the foundation for a pediatric clinical research system that is collaborative and sustainable. It is driven by scientific merit, produces clinically relevant medicines and devices, embraces the highest ethical standards and inspires others to support innovative clinical research for children.

OUR VALUES

- Children's unmet medical needs drive our agenda and all of our work supports the development of innovative medicines and devices for children that are most needed to improve and preserve children's health and wellness.
- We are an independent voice in innovative medicines and devices development, focused on optimal impact on child health.
- We are collaborative and synergistic with other elements of the clinical research ecosystem – finding common ground and aligning common values, with children at the center.
- Our standards are grounded in regulatory requirements, providing the basis upon which we work with others to deliver scientifically strong, clinically relevant and ethically sound advice and manage high-quality, timely conduct of clinical trials.
- We are focused on creating actionable data – meaning data that can be utilized to improve the safe and effective use of therapies, support robust regulatory submissions and enhance the information used by pediatric healthcare providers.
- Because children are waiting, we must shorten the time it takes to bring innovative medicines and devices to them. Streamlining operations, reducing burdensome processes and optimizing the use of resources are fundamental principles that guide us.

INSTITUTE FOR ADVANCED CLINICAL TRIALS FOR CHILDREN

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