INSTITUTE FOR ADVANCED CLINICAL TRIALS FOR CHILDREN

OCTOBER 2024

Welcome to the IACT4C Newsletter!

Content

News from IACT4C

PICTR Manuscript Published

The Pediatric Improvement Collaborative for Clinical Trials & Research (PICTR) was created to measure timelines and address delays in the pediatric clinical trials process.

Significant delays exist in industry-sponsored pediatric clinical drug trial start-up and enrollment processes

Authors: Angie Price (IACT4C), Hannah Simmons (Spark Excellence), Emily Gehring (Cincinnati Children's Hospital), Lauren Davis (Le Bonheur Children's Hospital), Gwyneth Fischer (University of Minnesota Masonic Children's Hospital), Ann R. Klipsch (Riley Hospital for Children), Erin Richmond (Driscoll Children's Hospital), Janice E. Sullivan (University of Louisville and Norton Children's Hospital), Steven J. Steiner (Riley Hospital for Children)

FDA Public Workshop "ADEPT-9: Enhancing Diversity in Therapeutics Development for Pediatric Patients"

IACT4C was privileged to attend a hybrid public workshop hosted by the FDA and the University of Maryland Center of Excellence in Regulatory Science and Innovation.

The workshop featured presentations and panel discussions from experts in the field, including researchers, regulators, industry representatives, and patients and families. Topics covered included the importance of diversity in clinical trials, challenges and opportunities for improving diversity in pediatric drug development, and strategies for involving diverse populations in research.

One key takeaway from the workshop was the importance of considering diversity in all aspects of drug development, from initial study design to data analysis and interpretation. By including diverse populations in clinical trials, researchers can better understand how different groups of patients respond to treatments and tailor therapies to individual patient needs.

Overall, the workshop was a valuable opportunity to learn from experts in the field and exchange ideas on improving diversity in therapeutic development for pediatric patients. IACT4C is committed to promoting diversity and inclusion in clinical research, and events like the ADEPT-9 workshop are essential for advancing this important work. We look forward to continuing our participation in initiatives supporting diversity in pediatric drug development and ultimately improving patient outcomes.

Hot Topics in Pediatric Drug Development

FDA issues Draft Guidance Providing New Details on Diversity Action Plans Required for Certain Clinical Studies.

The FDA issued a draft guidance "*Diversity Action Plans to Improve Enrollment of Participants from Underrepresented Populations in Clinical Studies*" to assist medical product sponsors in submitting Diversity Action Plans to support certain clinical.

Diversity Action Plans to Improve Enrollment of Participants from Underrepresented Populations in Clinical Studies Guidance for Industry

Harmonizing Quality Improvement Metrics Across Global Trial Networks to Advance Paediatric Clinical Trials Delivery

This paper aimed to define a common set of metrics that can be interchangeable/interoperable between global pediatric research networks from different jurisdictions by comparing various stages of study start-up metrics. It became apparent that comparing metrics was challenging due to contrasting study start-up data point definitions. Once there are shared definitions, common challenges and ways to improve study start-up processes can be explored.

Harmonizing Quality Improvement Metrics Across Global Trial Networks to Advance Paediatric Clinical Trials Delivery

Authors: Sabah Attar(c4c), Angie Price (IACT4C), Collin Hovinga (C-Path), Breanne Stewart (MICYRN), Thierry Lacaze-Masmonteil (MICYRN), Fedele Bonifazi (c4c), Mark A. Turner (c4c), Ricardo M. Fernandes(c4c)

Regulatory Processes for Rare Disease Drugs in the United States and European Union: Flexibilities and Collaborative Opportunities

NATIONAL ACADEMIES Sciences Engineering Medicine

Interesting new publication on drug development for rare diseases.

Regulatory Processes for Rare Disease Drugs in the United States and European Union: Flexibilities and Collaborative Opportunities

FDA Launches Rare Disease Innovation Hub

The Rare Disease Innovation Hub (the Hub) will work across rare diseases but will especially focus on products intended for smaller populations or for diseases where the natural history is variable and not fully understood.

FDA Rare Disease Innovation Hub to Enhance and Advance Outcomes for Patients

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What's New in Pediatric Regulatory

New FDA Guidances

Conducting Clinical Trials with Decentralized Elements

As part of a multifaceted effort by FDA to help modernize clinical trial design and conduct to improve efficiency and reduce burden on participants and on those conducting the trial, FDA issued a final guidance on decentralized clinical trials (DCT).

Conducting Clinical Trials with Decentralized Elements

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FDA Guidance Provides New Details on Diversity Action Plans Required for Certain Clinical Studies | FDA

SITE NETWORK UPDATES

Mentorship Registration is OPEN!

We are thrilled to announce the 2025 Mentorship Program! As we head into our fifth year, the Program has consistently proven to be a valuable resource for the pediatric clinical trial research community. It is designed to support and expand the current and next generation of researchers. We encourage all those interested in being a Mentee or Mentor to seize this invaluable opportunity and register now.

We look forward to working with you in 2025. For more information, please get in touch with the Site Network Team.

For IACT4C Site Network Members: **Register - Mentorship Program**

Educational Opportunities

I-ACT Educational Webinar Series

Save the Date!

Join us to learn about cell and gene therapy trial designs, unique risks and technologies used in CGT, challenges with scaling CGT and the ongoing ethical considerations.

December 10, 2024, from noon to 1pm ET

Register - IACT4C Webinar

Upcoming Conferences

MAGI@home brings best-in-class, accredited training and education from the comfort of your home or office.

Oct. 21-25, 2024 **Register**

AI and Machine Learning in Pediatric Research

Johns Hopkins All Children - In person or virtual

Jan 29, 2025 **Register**

SCOPE Summit

Summit for Clinical OPS Executives - In person or virtual

Feb 3-6, 2025 **Register**

News from Our Collaborators

The Children's Advisory Network (iCAN) is excited to share their Inaugural ImpactFall 2024 Fundraiser.

See the Flyer for more information: iCAN ImpactFall 2024 Fundraiser

To learn more about iCAN visit their website: [iCAN Website]

New Coordinator Corner

We would like to share some great resources with our unsung heroes of pediatric clinical research, the study coordinators!

Digital Handbook to Understand Advanced Therapies for Rare Diseases

Teams across the Sydney Children's Hospital Network (which is made up of two I-ACT for Children Network Sites: Sydney Children's Hospital and The Children's Hospital at Westmead) have come together to build a digital handbook to help patients and families understand advances therapies.

Co-designed by parents, clinicians, advocacy group representatives and researchers, the handbook houses a range of resources including videos, FAQs, checklists and more.

We thought you might find this helpful when you talk to your patients and families.

Digital Handbook Advanced Therapies

Free FDA Webinars Sponsored by the Division of Drug Information for 1-hr "Home Study" CME/AAPA/CNE/CPE/CPT/CPH

New on-demand "Home Study" CE Webinars!

Please visit our new and improved website!

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