INSTITUTE FOR ADVANCED CLINICAL TRIALS FOR CHILDREN

MAY 2024 NEWSLETTER

HOT TOPICS IN PEDIATRIC DRUG DEVELOPMENT

CDER Launches CDER Center for Clinical Trial Innovation (C_3TI)

As part of an ongoing effort to innovate and enhance clinical trials, CDER is establishing the CDER Center for Clinical Trial Innovation (C_3TI). C_3TI will foster CDER's innovation efforts and act as a central point for coordinating, sharing knowledge, and communicating with internal and external parties.

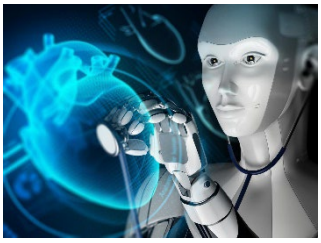
CDER Launches a Center for Clinical Trial Innovation | FDA

Why Patient Centricity is the Answer to the Clinical Trial Enrolment Gap

Research indicates that designing clinical trials with the patient in mind reduces recruitment times and enhances trial performance. Daniel J. Herron, a life sciences specialist, explains why document readability is one of the key factors in improving participant involvement.

https://www.pharmaceutical-technology.com/sponsored/why-patient-centricity-is-the-answer-to-the-clinical-trial-enrolment-gap/

FDA Publishes Artificial Intelligence Paper



FDA published its new paper, Artificial Intelligence and Medical Products: How CBER, CDER, CDRH, and OCP are Working Together, which outlines specific focus areas regarding the development and use of AI across the medical product lifecycle.

The multi-center effort from the FDA's Center for Drug Evaluation and Research, Center for Devices and Radiological Health, Center for Biologics Evaluation and Research, and the Office of Combination Products supports approaches to the safe, secure, ethical, and trustworthy development and use of AI.

The paper reflects the agency's commitment to prioritize collaboration, consistency, and mutual learning in this space. It also aims to promote responsible innovation, health equity, and patient-centricity by facilitating the secure, safe, ethical, and effective deployment and use of AI in medical products and in their development.

The paper will help further align and streamline the agency's work in AI. Read more about the agency's AI initiatives website.

Getting a Handle on Adverse Event Underreporting: Machine Learning to Drive Clinical Trial Efficiency via Adverse Event Data

White Paper: Integrated Clinical Solutions platform provides data analytics and visualizations for rapid interpretation of study performance and Predictions

Machine Learning to Drive Clinical Trial Efficiency via Adverse Event Data and Predictions

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Collection of Race and Ethnicity Data in Clinical Trials

Although uncommon, differences in response to medical products have been observed in racially and ethnically distinct populations in the United States. In some cases, differences in the pharmacokinetics, efficacy, or safety of medical products that lead to these different responses may be attributable to intrinsic factors (e.g., genetics, metabolism, elimination, skin pigmentation), extrinsic factors (e.g., diet, environmental exposure, socioeconomic status, culture), or interactions between these factors. Collecting data on race and ethnicity is critical to identify population-specific signals.

Collection of Race and Ethnicity Data in Clinical Trials and Clinical Studies for FDA-Regulated Medical Products | FDA

WHAT'S NEW IN PEDIATRIC REGULATORY

Informed Consent

To improve the informed consent process, the FDA announced the availability of a draft guidance entitled "Key Information and Facilitating Understanding in Informed Consent." This draft guidance provides recommendations related to two provisions of the revised Federal Policy for the Protection of Human Subjects (the revised Common Rule) by the U.S. Department of Health and Human Services (HHS) and identical provisions in the FDA's proposed rule "Protection of Human Subjects and Institutional Review Boards." FDA's proposed rule, if finalized, would harmonize certain sections of FDA's regulations on human subject protections and institutional review boards (IRBs), to the extent practicable and consistent with other statutory provisions, with the revised Common Rule, in accordance with the 21st Century Cures Act. The guidance addresses the provisions of the revised Common Rule that require informed consent to begin with key information about the research and to present information that facilitates understanding and identical provisions in the FDA's proposed rule.

EDUCATIONAL OPPORTUNITIES

SAVE THE DATE!
June 25th @ 12pm ET

Join us in exploring how cell and gene therapy (CGT) is revolutionizing pediatric drug development and offering new hope for treating genetic disorders. This webinar will provide a general understanding of the CGT process, the requirements to conduct clinical trials, the regulations, and the challenges, risks, and benefits of participating in pediatric CGT trials.

Don't miss this exclusive opportunity to delve into the world of pediatric CGT and learn from the industry's top leaders. Whether you're a seasoned professional or new to CGT, this webinar will equip you with practical insights and strategies you can apply immediately in your work, preparing and conducting CGT trials.

Meet the experts:

Featured Speakers

Conrad Russel Y. Cruz, M.D., Ph.D., Principal Investigator, Program for Cell Enhancement and Technologies for Immunotherapies, Children's National Hospital

Daniel Eisenmann, Ph.D., CBSP, Executive Director Biosafety Services, Advarra

Sarah McCague, MS, Administrative Manager of Clinical In Vivo Gene Therapy, Children's Hospital of Philadelphia

All are welcome to attend this free event. Registration is required to reserve your seat.

Register Now

Upcoming Conference:

APP: Practical Care of the Adolescent and Young Adult Course
Denver, CO – May 16-19, 2024
2024 Practical Care of the Adolescent and Young Adult Course
AAP: CardioPREP: An Intensive Review and Update of Pediatric Cardiology
Virtual – July 18-21, 2024
2024 CardioPREP: An Intensive Review and Update of Pediatric Cardiology

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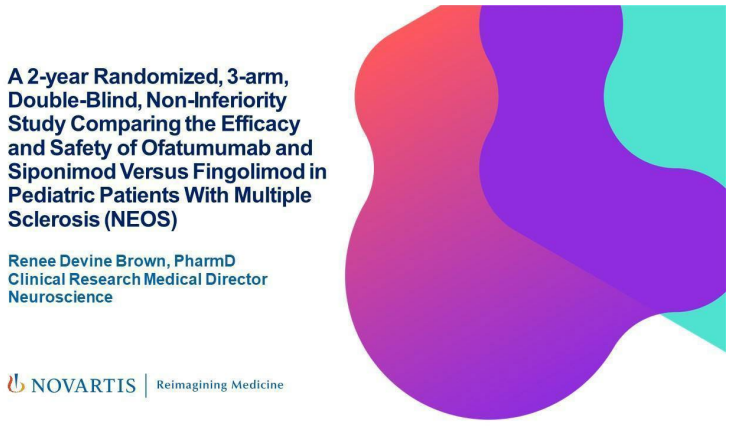
SITE NETWORK UPDATES

Thank you for your participation in the oncology capabilities survey! Your input is invaluable as we explore potential study opportunities in the field of oncology and hematology. Should any such opportunity arise, we will promptly reach out to assess your interest.

Opportunity to Learn More About a Pediatric MS Study!

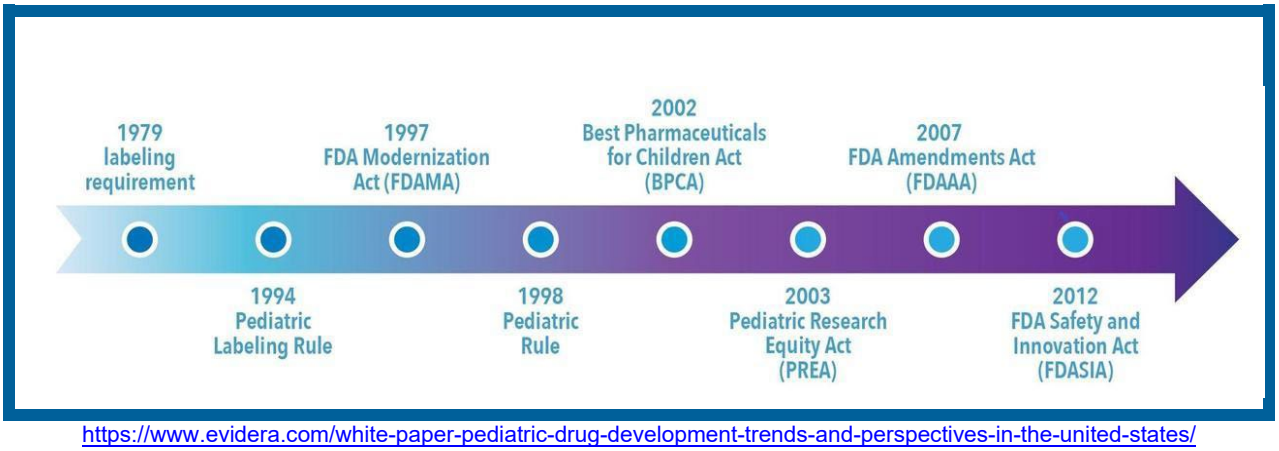


- Recorded Presentation: Novartis Pediatric MS Study (NEOS) - Introduction (Confidential)
- Slide Deck: Novartis Pediatric MS Study (NEOS)

- If you want to learn more, please contact the Clinical Project Manager: Azra Qizilbash (azra.qizilbash@novartis.com) or the Clinical Research Medical Director, NS Portfolio: Dr. Renee Brown (renee.brown@novartis.com).

WELCOME TO THE NEW "COORDINATOR CORNER"

Understanding that many of you are new to pediatric research, we want to start out with a high-level overview of the historical milestones and legislation that have shaped pediatric drug development. These key events have significantly impacted how medications are studied and labeled for use in children:



https://www.evidera.com/white-paper-pediatric-drug-development-trends-and-perspectives-in-the-united-states/

Links and Abbreviations

Links:

- FDA Overview of the Pediatric Legislation by Dr. Susan McCune

- Podcast: Why Pediatric Clinical Trials Need to Grow Up by Dr. Cindy Jackson
- BPCA: About BPCA - BPCA | NICHD
- FDASIA: Fact Sheet: Pediatric provisions in the Food and Drug Administration Safety and Innovation Act
- RACE for Children Act: Here's What Sponsors Need to Know About the Race for Children Act
- Federal Policy for the Protection of Human Subjects ('Common Rule') Federal Policy for the Protection of Human Subjects ('Common Rule | HHS.gov
- Revised Common Rule Information: HHS: Revised Common Rule
- Comparison of FDA and HHS Human Subject Protection Regulations:
- https://www.fda.gov/science-research/good-clinical-practice-educational-materials/comparison-fda-and-hhs-human-subject-protection-regulations
- FDA Code of Federal Regulations:
- 21 CFR Part 56 (Protection of Human Subjects) FDA: 21 CFR Part 56
- 21 CFR Part 50 (Protection of Human Subjects) FDA: 21 CFR Part 50
- E11(R1) Addendum: Clinical Investigation of Medicinal Products in the Pediatric Population
- E11(R1) Addendum: Clinical Investigation of Medicinal Products in the Pediatric Population | FDA

Abbreviations:

Abbreviation	Name	Abbreviation	Name
AAP	American Academy of Pediatrics	**OOPD**	Office of Orphan Products Development
BPCA	Best Pharmaceuticals for Children Act	**OPT**	Office of Pediatric Therapeutics
FD&C Act	Federal Food, Drug, and Cosmetic Act	**PTUDFA**	Prescription Drug User Fee Amendments
FDAMA	FDA Modernization Act	**PeRC**	Pediatric Review Committee
FDASIA	Food and Drug Administration Safety and Innovation Act	**PRFA**	Pediatric Research Equity Act
NICHD	National Institute of Child Health and Human Development	**RACE**	Research to Accelerate Cures and Equity (RACE) for Children Act

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