CENTER FOR DRUG EVALUATION AND RESEARCH

Center For Clinical Trial Innovation (C3TI)

Welcome to the Spring 2026 issue of the CDER Center for Clinical Trial Innovation (C3TI) newsletter. We are excited to share updates on C3TI's efforts to foster clinical trial innovation. Please reach out to us at cderclinicaltrialinnovation@fda.hhs.gov with feedback or questions.

C3TI News

Register Today: C3TI to Host a Public Workshop on Clinical Trial Innovation

This workshop will explore progress in clinical trial innovation to improve efficiencies in drug development. The April 14 meeting will highlight C3TI's activities, including the C3TI Demonstration Program, and emerging priorities in novel clinical trial approaches. Sessions will discuss lessons learned from recent efforts, the benefits of adopting innovative approaches in clinical trials, and persistent challenges. Panelists will discuss strategies to bridge from policy to implementation to broader adoption of innovative approaches in the design and conduct of clinical trials.

C3TI Updates its Knowledge Repository, C3TI Compass

C3TI updated and republished its knowledge repository, C3TI Compass. C3TI Compass connects users to FDA guidance documents, case studies, and resources that support innovative approaches to clinical trial design and conduct. It centralizes completed activities, ongoing efforts, and practical tools and organizes resources by topic area and resource type.

C3TI and TransCelerate Engage in 'Tabletop' Exercise on Safety Reporting in Pragmatic Trials

C3TI and TransCelerate BioPharma held a "tabletop" exercise in October 2025 focused on safety reporting in clinical trials with pragmatic elements. By considering simulated scenarios across multiple therapeutic areas, participants identified ways to address Selective Safety Data Collection (SSDC) implementation barriers and support sponsors in their drug development programs. Participants discussed adaptive plans for switching between SSDC and comprehensive data reporting in an ongoing trial, incorporation of SSDC into pre-approval trials, global alignment challenges, and use of electronic health records for data collection. A public summary will be available soon.

C3TI Training Needs Assessment on Clinical Trial Innovation

C3TI is conducting a training needs assessment to strengthen education and knowledge-sharing around clinical trial innovation. This assessment will help C3TI align its internal and external training and communication efforts with the needs of the clinical trial innovation community. C3TI recognizes the importance of providing CDER review staff and external parties with clear, accessible, and practical training resources to help bridge the gap between policy and real-world application. We look forward to sharing updates as the assessment findings inform future C3TI efforts.

C3TI 'Champion' Co-Authors Two Articles About Artificial Intelligence in Drug Development

Atasi Poddar, PhD, a health science policy analyst in CDER's Office of Medical Policy and C3TI Champion, recently co-authored two papers on the use of artificial intelligence (AI) in drug development. One article provides a summary of an FDA workshop on guiding principles, challenges, and best practices for the responsible use of AI in drug development. The other article summarizes discussions from the same workshop on how AI could be leveraged to mitigate limited data challenges in rare disease drug development.

C3TI Champions are CDER staff who partner with C3TI to help promote and nurture a culture of clinical trial innovation.

FDA Clinical Trial Innovation News

FDA Publishes Guidance on Bayesian Methodology in Clinical Trials

FDA published the draft guidance, Use of Bayesian Methodology in Clinical Trials of Drug and Biological Products, designed to facilitate the use of Bayesian methodologies in clinical trials of drug and biological products, helping drug developers make better use of available data, conduct more efficient clinical trials, and deliver safe and effective treatments to patients sooner. The guidance emphasizes the use of Bayesian methods to support primary inference in a clinical trial. Bayesian Statistical Analysis is one of three projects in C3TI's Demonstration Program.

FDA and EMA Publish Guiding Principles of Good AI Practice in Drug Development

CDER and the Center for Biologics Evaluation and Research (CBER) collaborated with the European Medicines Agency to develop 10 guiding principles that industry and product developers can consider when using AI to advance drug and biological product development. This new resource outlines a common set of principles to inform, enhance, and promote the use of AI for generating evidence across all phases of the product life cycle.

FDA Qualifies Two Drug Development Tools to Expedite Drug Development

At the end of 2025, FDA qualified two Drug Development Tools (DDTs) that will help expedite drug development. The AI-Based Histologic Measurement of NASH (AIM-NASH) is the first qualified AI DDT. AIM-NASH will help pathologists assess metabolic dysfunction-associated steatohepatitis (MASH) disease activity in clinical trials by scoring liver biopsy components.

FDA also qualified total hip bone mineral density (BMD) as assessed by dual energy X-ray absorptiometry as a validated surrogate endpoint to support clinical trials of investigational therapies for post-menopausal women with osteoporosis at risk for fracture. The BMD DDT is intended to foster more efficient clinical trials, potentially enabling faster approval of new osteoporosis treatments, and improving patient access.

DDTs play an important role in bringing new therapies to patients by providing well-defined, scientifically sound approaches to clinical trial design and regulatory decision-making.

FDA/MHRA-UK/Health Canada Symposium Will be Held in June

The FDA/Medicines and Healthcare products Regulatory Agency (MHRA)-UK/Health Canada Symposium, will be held June 2-4, 2026, in Ottawa, Canada, with the option to join virtually. This event will bring together regulators, investigators, clinical researchers, clinical trial staff, sponsors, research organizations, service providers, pharmaceutical and biotechnology companies, academics and patient advocacy groups. Participants will discuss good clinical practice, bioequivalence, good pharmacovigilance practice, ICH E6(R3) implementation, innovative trials, case studies, and more.

FDA Approves First Treatment for Children with Menkes Disease; Drug was Evaluated in Two Open-Label, Single-Arm Clinical Trials

FDA approved Zycubo (copper histidinate) injection as the first treatment for Menkes disease in pediatric patients. Menkes disease, a rare neurodegenerative disorder caused by a genetic defect that impairs a child's ability to absorb copper, is characterized by seizures, failure to gain weight and grow, developmental delays, intellectual disability, and premature death.

FDA evaluated Zycubo in two open-label, single-arm clinical trials in pediatric patients. Investigators assessed overall survival by comparing treated patients to untreated patients from contemporaneous external control groups. Children who began treatment within four weeks of birth had a 78% reduction in the risk of death compared with untreated patients. Nearly half of early-treated patients survived beyond six years, and some survived more than 12 years. No patients in the untreated control group survived beyond six years. Children who started treatment later than four weeks after birth also experienced a substantial survival benefit.

FDA Hosts Rare Disease Day Virtual Public Meeting on Feb. 23

FDA hosted Rare Disease Day on Feb. 23, 2026, in global observance of Rare Disease Week. This year's theme was "Moving Forward. Looking Ahead. An Event for Patients." The goal of Rare Disease Day was to explore ways to engage and collaborate with patients and their communities to support and accelerate the development of medical products for rare diseases. Panels discussed patient-focused FDA initiatives, patient

engagement opportunities, addressing challenges and opportunities with AI technology, and utilizing real-world data and real-world evidence to support approval of products for rare diseases

CDER Publishes 2025 Novel Drug Approvals Report

CDER released its 15th annual report, Advancing Health Through Innovation: New Drug Therapy Approvals 2025, summarizing the 46 novel drug and biological product approvals of the past calendar year. Seventy percent of drugs (32) were approved in the U.S. before approval in other countries, and exactly half (23) received orphan drug designation. The number of novel product approvals in 2025 was similar to the average for the last five years, and above the historical average of 38 novel products per year since 2007. Combined with the CBER, there were 58 novel approvals in 2025.

ICYMI: C3TI on the Road

C3TI leadership recently spoke at:

- 1/11/2026: UCSF-Standard Center of Excellence in Regulatory Science and Innovation (CERSI) 7th Annual Innovations in Regulatory Science Summit
- 2/4/2026: SCOPE Summit for Clinical Operations Executives
- 2/20/2026: Decentralized Trials Research Alliance Podcast on Selective Safety Data Collection

Interested in requesting a C3TI speaker? Submit a CDER Speaker Request here.
