



CENTER FOR DRUG EVALUATION AND RESEARCH

Center For Clinical Trial Innovation (C3TI)

Welcome to the latest issue of the CDER Center for Clinical Trial Innovation (C3TI) newsletter. This newsletter aims to provide you with updates on our work to promote innovation in clinical research. Questions or feedback? Email us at CDERclinicaltrialinnovation@fda.hhs.gov.

C3TI News

C3TI Publishes Frequently Asked Questions about Its Demonstration Program

Based on stakeholder feedback and to improve clarity about the initiative, C3TI has published a set of [questions-and-answers about its Demonstration Program](#). These questions include general items about who is involved in the program and what benefits participants can expect. There are also practical questions about how to submit a proposal and what the proposal review process entails.

Upcoming Webinar about Navigating the FDA Bayesian Draft Guidance

FDA will host a [webinar on October 14 to help stakeholders navigate the January 2026 draft guidance](#), "Use of Bayesian Methodology in Clinical Trials of Drug and Biological Products." The webinar will include an introduction to Bayesian approaches, success criteria and operating characteristics in Bayesian trials, and considerations for prior distributions. [C3TI Champion](#) James Travis, PhD, will be a presenter.

Workshop Summary Available from the C3TI-Duke Margolis Workshop on Scaling Innovative Clinical Trial Approaches

Materials, including a workshop summary, are available from the C3TI-Duke Margolis Workshop, ["Scaling Innovative Clinical Trial Approaches: Challenges, Progress, and Opportunities."](#) This April 14 workshop explored progress in clinical trial innovation to improve efficiencies in drug development. Panelists discussed C3TI's activities and emerging priorities in novel clinical trial approaches. Participants noted that broader adoption of innovative clinical trial approaches requires action across global regulatory alignment, operational standardization, and knowledge-sharing. C3TI is incorporating the feedback from the workshop into its ongoing activities and programs.

Unified Agenda Publishes Notice of FDA's Upcoming Proposed Rule Revising Responsibilities of Sponsors and Investigators for Clinical Investigations of Drugs and Biologics

The Unified Agenda has published [FDA's intention to put forth a proposed rule](#) that aims to revise the responsibilities of sponsors and investigators for the conduct of clinical investigations of drugs and biologics. This rule would update the agency's requirements to reflect the modern clinical trial ecosystem with a focus on clinical trial quality. The rule, when implemented, would improve FDA's ability to ensure the rights, safety, and welfare of trial participants and the reliability of results while supporting innovation and efficiency in clinical trial conduct. In addition, the proposed updates would align FDA's requirements with those of our global regulatory counterparts.

The Unified Agenda reports on the actions administrative agencies plan to issue in the near and long term. Released by the Office of Management and Budget, the Unified Agenda provides public notice and transparency about proposed regulatory and deregulatory actions within the Executive Branch.

C3TI "Champion" Reflects on the Importance of Innovating Clinical Trial Enrollment

In honor of Clinical Trials Day, [C3TI published a first-hand reflection](#) of why Mitchell Psotka, MD, PhD, is committed to innovating clinical trial enrollment. Dr. Psotka, a Medical Officer in CDER's Office of New Drugs and a [C3TI Champion](#), explained that his time treating and counseling patients about cardiovascular clinical trials inspired him to want to make trials more efficient. Hearing about enrollment challenges drew Dr. Psotka to C3TI and specifically to programs like Selective Safety Data Collection, one of C3TI's Demonstration Project Areas where he now serves as a co-lead.

FDA Clinical Trial Innovation News

FDA Publishes a White Paper on Digitally Derived Measures for Clinical Investigations

FDA's medical product centers have published a white paper, "[Key Considerations for the Development and Use of Digitally Derived Measures for Clinical Investigations.](#)" The white paper highlights key considerations drawn from FDA guidances for the development and use of digitally derived measures (DDMs) as outcomes in clinical investigations. DDMs are measures derived from data collected using digital health technologies. DDMs may serve as clinical outcome assessments, biomarkers, or components of composite endpoints.

ICH M11 Clinical Electronic Structured Harmonised Protocol Now Available on FDA Website

FDA has published the [International Council for Harmonisation \(ICH\) M11 Clinical Electronic Structured Harmonised Protocol](#), which establishes the first internationally harmonized standard for the format, content, and electronic exchange of clinical trial protocols. The guidance, adopted by the ICH Assembly in November 2025, includes a standardized Protocol Template and Technical Specification applicable to all phases of interventional clinical trials across therapeutic areas.

This guidance supports FDA's clinical trial modernization efforts, commitment to international harmonization through ICH, and broader goals of improving drug development efficiency. FDA played a significant leadership role in developing this guidance. The next implementation steps include developing training materials and determining FDA's standard for data exchange of protocol information.

FDA Accepts First In Silico Drug Development Tool Under ISTAND to Help Predict Drug-Induced Liver Injury

FDA has accepted the first Letter of Intent (LOI) for an [in silico drug development tool \(DDT\)](#) into the Innovative Science and Technology Approaches for New Drugs (ISTAND) DDT Qualification Program. The DDT is an Artificial Intelligence Driven Digital Liver Model for Prediction of Drug-Induced Liver Injury. It aligns with FDA's vision of optimizing drug development and evaluation while minimizing the use of animals in the nonclinical space, potentially expediting the availability of safe and effective treatments.

FDA Accepts Wearable Sensor-Derived Foot Drop Metrics Under ISTAND as Longitudinal Outcome Measures in Rare Neurological Condition

ISTAND has accepted an LOI for a [wearable sensor-derived digital outcome measure](#) intended to quantify foot drop severity and its impact on gait in adult patients with Charcot-Marie-Tooth disease type 1A, a progressive, degenerative disease. This LOI represents the first submission to be accepted into ISTAND for a digital health technology for a rare neurological condition.

FDA Releases Updated Public Report on RWE Submissions in CDER and CBER

FDA released an updated public report with Fiscal Year 2026 data on real-world evidence (RWE) submissions to the [Center for Biologics Evaluation and Research \(CBER\)](#) and [CDER](#). This report highlights submissions that contain RWE and meet established reporting criteria, fulfilling an agency commitment under the Prescription Drug User Fee Act.

FDA Publishes 73 Examples of Medical Device Marketing Authorizations using RWE

[FDA published a new set of 73 examples of marketing authorizations for medical devices using RWE.](#) These examples, which cover FY 2020-2025, represent a broad spectrum of clinical and device areas throughout FDA's Center for Devices and Radiological Health (CDRH). While registries have played a central role for a long time, other sources — such as electronic health records aggregators, administrative claims and billing data, device-generated data, digital health platforms, and increasingly sophisticated data linkages — are contributing to a broader evidence environment.

FDA Seeks Public Feedback to Inform Regulatory Approach for Generative AI-Enabled Medical Devices

[FDA has issued a discussion paper on considerations for the regulation of generative artificial intelligence \(GenAI\)-enabled medical devices](#), seeking feedback from parties on risk assessment, premarket evaluation, postmarket monitoring, and other relevant topics. GenAI-enabled medical devices hold promise for patient care and the broader health

ecosystem, but they may introduce risks compared to traditional software and AI-enabled medical devices.

FDA Issues Draft Guidance to Help Accelerate Cell and Gene Therapies for Patients

[FDA issued draft guidance to help developers bring promising gene therapies to patients more efficiently by making greater use of existing scientific and regulatory knowledge.](#)

When finalized, the guidance will outline how sponsors can use public information and established platform knowledge — including chemistry, manufacturing and controls data, nonclinical study results and clinical information — to streamline regulatory submissions for human gene therapy products that use genome editing in human somatic cells.

FDA Advances Drug Repurposing to Address Unmet Medical Needs

FDA is [soliciting input on efforts with respect to drug repurposing to help address unmet medical needs across a range of diseases and conditions](#). Identifying potential new uses — such as a new indication or a new population — for FDA-approved drugs can help accelerate the availability of treatments by using existing knowledge about the drugs, including a drug's safety profile.

CDER ARC's LEADER 3D Announces New Rare Disease Drug Development Educational Materials

FDA's CDER [published six new case studies and a video that were developed as part of the Accelerating Rare disease Cures \(ARC\) Program's Learning and Education to ADvance and Empower Rare Disease Drug Developers \(LEADER 3D\) initiative](#). These materials highlight approaches sponsors successfully used when designing and conducting rare disease drug development programs.

RDEA Pilot Program Workshop Materials Available

[Recording and slides are available from a workshop convened by FDA and Triangle Center of Excellence in Regulatory Science and Innovation on May 18](#) that focused on the development of surrogate endpoints for drugs and biological products intended to treat rare diseases. At this Rare Disease Endpoint Advancement (RDEA) Pilot Program workshop, participants discussed mechanistic and translational science evidence needed to support use of novel surrogate endpoints in rare disease marketing applications. Discussion also included new approach methodologies, digital health technologies, and harnessing varied sources of patient-level real-world data.

ICYMI: C3TI on the Road

C3TI leadership recently spoke at:

- 6/2: FDA/Medicines and Healthcare Products Regulatory Agency/Health Canada Symposium
- 6/11: National Institute of Allergy and Infectious Diseases Office of Clinical Research Policy and Regulatory Operations' 2026 Learning Event
- 6/15-6/16: Drug Information Association Global Annual Meeting

- 6/23: Biotechnology Innovation Organization International Convention
- 7/14/-7/15: National Academies' Forum on Drug Discovery, Development, and Translation: Workshop on Engaging Community-Based Practices in Clinical Trials
- 9/17: American Statistical Association Biopharmaceutical Section Regulatory-Industry Statistics Workshop: Developing the FDA Bayesian Guidance
- 9/23: Pioneering the Path for Rare Disease: A Transatlantic Policy and Innovation Summit
- 9/23: Association of Clinical Research Organization's Inaugural Global Clinical Trials Regulatory Summit

Interested in requesting a C3TI speaker? Submit a [CDER Speaker Request](#) here.

Questions or Comments? Contact C3TI at cderclinicaltrialinnovation@fda.hhs.gov.

To learn more about C3TI, visit the [C3TI homepage](#).

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