

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. C3TI has been engaged in many important projects this spring and summer, and we are excited to provide you with an update. Please reach out to us at [cderclinicaltrialinnovation@fda.hhs.gov](mailto:cderclinicaltrialinnovation@fda.hhs.gov) with feedback or questions.

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## C3TI News

### **C3TI Issues External White Paper on Selective Safety Data Collection**

C3TI has issued a [white paper](#) focused on selective safety data collection (SSDC), which is the planned reduction in the collection of certain types of data in a clinical investigation for drugs with a well-characterized safety profile. The white paper describes the International Council for Harmonization's E19 guidance, A Selective Approach to Safety Data Collection in Specific Late-Stage Pre-approval Approval or Post-approval Approval Clinical Trials, which FDA and other regulatory agencies have implemented.

The white paper discusses trials that have incorporated SSDC, including in the oncology (Pragmatica-Lung Study) and cardiometabolic spaces (VICTORION-2-PREVENT, EMPA-KIDNEY, and EMPACT-MI). It also looks to the future, seeing how the clinical trial ecosystem can expand the use of SSDC through C3TI's Demonstration Program and other efforts.

### **C3TI's Bayesian Demonstration Project Broadens Its Focus**

[C3TI's Bayesian Statistical Analysis \(BSA\) demonstration project](#) has expanded its scope to include pre-specified primary analysis and trial monitoring in addition to its previous focus on supplementary analysis. The project remains interested in phase 3 efficacy or safety trials with simple designs, such as trials with non-adaptive or straightforward sequential designs.

C3TI expanded the BSA demonstration project based on internal and external feedback, and to encourage further sponsor participation. It is one of three projects in [C3TI's Demonstration Program](#).

### **C3TI 'Champion' Co-Authors 2 Papers on Clinical Trial Innovation**

Cheryl Grandinetti, a C3TI Champion who works in CDER's Office of Compliance, recently co-authored two articles on clinical trial innovation. [One article](#) discusses how FDA uses good clinical practice inspections to verify the quality, integrity, and reliability of real-world data (RWD) in regulatory submissions. Through case examples, the article highlights challenges related to accessing RWD source records, assessing data quality, and evaluating processes for data curation, transformation, and analysis.

In [another article](#), Grandinetti and co-authors discuss opportunities for the integration and use of artificial intelligence (AI)/machine learning (ML) in clinical trials. This review explores the multifaceted landscape of AI/ML applications in clinical trials and includes use cases that aim to improve data quality and enhance patient outcomes and clinical trial successes.

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

## Spotlight: Streamlined Trials Embedded in clinical Practice (STEP)

Integrating aspects of clinical trials into routine care may offer many benefits, such as resource-efficiency, expediency, and a means to recruit more patients into clinical trials. With the [Streamlined Trials Embedded in clinical Practice \(STEP\) demonstration project](#), C3TI is engaging with sponsors planning point-of-care trials to help address and resolve issues (e.g., statistical analyses, incorporation of real-world data and evidence, trial endpoint selection, inspectional approaches) around trial design and conduct.

STEP leaders and other CDER staff participated in a "tabletop exercise" with TransCelerate BioPharma. This hypothetical exercise, focused on sponsor oversight, allowed participants to discuss and understand challenges that sponsors face when designing and conducting trials with pragmatic elements. In this tabletop exercise, individuals discussed issues such as capturing data in the electronic health record, streamlining safety data collection, and implementing new health technologies.

A [summary of the exercise](#) is available. Learn more about the [C3TI Demonstration Program](#).

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## FDA Clinical Trial Innovation News

**FDA, National Institutes of Health Issue Modernizing Research and Evidence (MoRE) Consensus Definitions**

FDA and the National Institutes of Health (NIH) have published a [glossary of consensus definitions](#) of common terminology used in clinical research. This document aims to facilitate communication within the clinical trial community. It defines types of trials, studies, models, errors, and other important terms. This document may help the research community better evaluate potential strengths and limitations of individual studies, and convey clinical research findings in a meaningful way to funders, reviewers, and the public.

### **FDA Publishes Final Guidance on Remote Regulatory Assessments**

FDA has published a [final guidance](#) on remote regulatory assessments (RRAs) to provide answers to frequently asked questions. In response to the Coronavirus Disease 2019 (COVID-19) pandemic, FDA adapted its operations for field activities. One set of tools the agency used was RRAs. Since that time, FDA has noted the value of RRAs and concluded that they are a beneficial oversight tool for a range of FDA-regulated products. As such, the agency developed a guidance to help enhance industry's understanding of RRAs. This guidance finalizes the revised draft guidance of the same title issued on Jan. 26, 2024.

### **ISTAND Transitions to Permanent Program**

FDA's [Innovative Science and Technology Approaches for New Drugs \(ISTAND\) Pilot Program](#) has transitioned to a [permanent program](#). The 21st Century Cures Act established a pathway for FDA to "qualify" certain drug development tools (DDTs), such as biomarkers, clinical outcome assessments, and animal models. In November 2020, FDA launched ISTAND as a pathway for novel DDTs that did not fit within existing qualification programs.

In its pilot phase, ISTAND helped facilitate the integration of advanced tools, such as organ-on-a-chip models, AI-enabled tools, digital health technologies, and complex in vitro models into regulatory science. As a fully operational qualification program, ISTAND will continue supporting innovative, science-driven approaches that improve drug development and regulatory decision-making.

### **CDER Releases Drug Trial Snapshots Summary Report**

CDER has released the [2024 Drug Trials Snapshots Summary Report](#), which summarizes the demographic representation of participants in the clinical trials for the 50 new drugs approved in 2024. The report provides information by sex, race, ethnicity, age group, and U.S. participation. Overall, about 31,000 patients participated in these trials.

## **ICYMI: C3TI on the Road**

C3TI leadership spoke at in-person local as well as virtual events, including the [Drug Information Association \(DIA\) Annual Meeting](#) in mid-June in Washington, D.C. At the DIA Annual Meeting, staff discussed aspects of innovation in clinical trials, such as integrating clinical trials in clinical care; encouraging health care professionals to engage in clinical

research; examining the intersection of data quality assurance and clinical innovation; and many other important topics.

Other recent C3TI speaking events include:

- 4/1/2025: DIA/FDA Statistical Forum
- 6/17/2025: Workshop on the use of Bayesian Statistics in Clinical Development
- 7/30/2025: Association of Clinical Research Professionals: FEAR: Face Everything and Rise (in Clinical Research)

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

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Questions or Comments? Contact C3TI at [CDERClinicalTrialInnovation@fda.hhs.gov](mailto:CDERClinicalTrialInnovation@fda.hhs.gov).

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