The CDER Center for Clinical Trial Innovation (C3TI)

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CDER Center for Clinical Trial Innovation

On April 15, 2024, CDER launched C3TI

to enable and amplify innovative approaches to clinical trials that are designed to improve the efficiency and effectiveness of drug development.

For years, CDER has championed clinical trial innovation

Key Programs

- Complex Innovative Trial Designs
- Digital Health Technologies
- Drug Development Tool Qualification
- Model-Informed Drug Development
- Patient-Focused Drug Development
- Rare Disease Endpoint Advancement
- Real-World Evidence

Additional Focus Areas

- Artificial intelligence and machine learning
- Decentralized clinical trial designs
- Enrollment of participants from underrepresented populations
- International harmonization
- Public-private partnerships and external collaborations
- Simpler trials that could more easily be integrated into clinical practice

These efforts have enhanced the design and conduct of clinical trials intended to generate evidence of safety and effectiveness of therapies.

CDER Center for Clinical Trial Innovation (C3TI)

Over the last year, we sought to better understand the impact of our clinical trial innovation efforts both internally across CDER and externally.

Interviews and listening sessions with CDER leadership and review staff

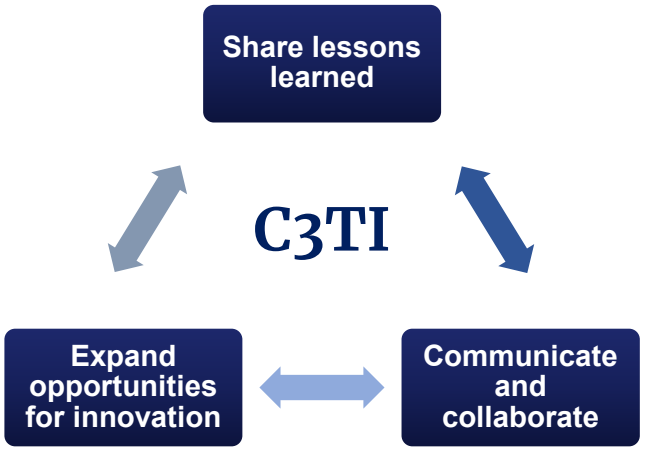
Interviews with industry, non-profits, patient advocacy groups, public-private partnerships and other interested parties

Public workshop and docket

Based on the information, we recognized an opportunity to enhance the implementation of our efforts and maximize the impact on drug development.

CDER Center for Clinical Trial Innovation (C3TI)

Mission: To promote existing and future CDER clinical trial innovation through enhanced communication and collaboration



Learn more

www.fda.gov/C3TI

CDERclinicaltrialinnovation@fda.hhs.gov

Key Activities:

- C3TI Compass, a centralized knowledge repository
- Internal and external communication and engagement
- Single point-of-contact for innovation-related questions
- Demonstration program to scale adoption of innovation

C3TI Compass

COMPASS

- A searchable platform for information about clinical trial innovation activities.
- Contribute to Compass to help maintain a comprehensive resource knowledge sharing.

Click here to access Compass.

Objectives

- Sharing knowledge, lessons learned, and tools on clinical trial innovation topics via a centralized knowledge repository known as C3TI Compass
- Curate credible and important information in an easy-to-access format
- Connect users quickly to information to help accelerate the pace of clinical trial innovation
- Promote a culture of contribution and collaboration to drive innovation as a behavior

C3TI Demonstration Program

Through **targeted projects to explore scalable improvements** in the adoption of select clinical trial innovations, the C3TI Demonstration Program:

- **Expands** opportunities for sponsors of innovative clinical trials to engage with CDER staff
- **Supports** trials that can serve as case examples to spur further implementation
- **Enhances** the existing body of knowledge and precedents for clinical trial innovations

Initial Project Areas

Bayesian Supplementary Analysis

Selective Safety Data Collection (SSDC)

Streamlined Trials Embedded in clinical Practice (STEP)

To learn more about the demonstration program, including proposal eligibility criteria and the submission process, visit www.fda.gov/C3TI.

C3TI Demonstration Program (II/IV)

Bayesian Supplementary Analysis

- Aims to **increase experience** in Bayesian statistical methods in simple trials settings among sponsors, CDER clinical reviewers, and CDER statisticians.
- C3TI will partner with sponsors to use Bayesian methods in supplementary analyses during their trial, providing an opportunity for both CDER and sponsors to **learn without impacting review** criteria.

Eligibility Criteria

- A phase 3 efficacy, safety, or non-inferiority standalone trial (i.e., not incorporating data from previous trials beyond informing the non-inferiority margin) with a simple non-adaptive design.
- The Bayesian analysis should supplement the primary analysis and may be used to evaluate the primary endpoint in the overall study population and/or in relevant subgroups (i.e., for subgroup analysis).

Additional information: Bayesian Supplemental Analysis (BSA) Demonstration Project

C3TI Demonstration Program (III/IV)

Selective Safety Data Collection (SSDC)

- **Streamline data collection** in late-stage pre-approval or post-approval trials, to reduce the burden of collecting unnecessary safety data, eliminate unnecessary expense, and facilitate trial conduct to answer important scientific questions on long-term efficacy and safety of drugs and biologics.
- Aims to **improve understanding of SSDC**'s real-world applicability, demonstration of its ability to facilitate efficient clinical trials, identification of potential challenges that programs encounter and ways to address those challenges.

Eligibility Criteria

- A late-stage pre- or post-marketing trial for a drug product where the safety profile, with respect to commonly occurring adverse events, is well understood and documented. This may include:
  - Trial for an approved drug, seeking a new indication in a similar population to that in which it was already approved
  - Trial for an approved drug, seeking to expand the label to include additional endpoints in the same patient population
  - Safety trial investigating a very specific safety concern (e.g., a PMR under FDAAA)
  - Trial designed to provide additional evidence of efficacy when current data support a well-characterized safety profile

Additional information: Selective Safety Data Collection (SSDC) Demonstration Project

C3TI Demonstration Program (IV/IV)

Streamlined Trials Embedded in clinical Practice (STEP)

- Aims to **promote the adoption of pragmatic design** in clinical trials and improve coordination and collaboration between CDER and sponsors to effectively support these innovative trials.
- These types of trials are advantageous as they can be **more resource-efficient**, able to better **attract broader study populations**, can be **completed more rapidly**, and yet **still robustly assess study objectives**.

Eligibility Criteria

- The trial incorporates pragmatic design elements that are reflective of routine clinical practice to improve trial efficiency and enhance patient centricity while maintaining patient safety and data integrity.
- The trial fits one of the following criteria:
  - Trial later in pre-market development, when the safety profile is reasonably well-defined.
  - Post-approval trial (either that the sponsor initiated or in response to a post-marketing requirement), wherein the population, trial procedures, and endpoints can all be appropriately incorporated into a large simple trial.

Additional information: Streamlined Trials Embedded in clinical Practice (STEP) Demonstration Project

C3TI Outcomes

Short term	Medium term	Long term
High **user satisfaction of resources** (e.g., case study library, internal symposia, communication hub)	Increase in # of drug development programs with **Sponsor-initiated clinical trial innovation** strategies	**Clinical trials** are more **efficient and accessible to patients** (while maintaining gold standard for high quality)
Parties **acquire skills** on the **latest innovations** in clinical trial design and conduct	Improved and more **streamlined communication and collaboration** for both internal and external stakeholders	**Amplify the impact** of ongoing and future CDER clinical trial **innovation initiatives**
Increased **awareness of novel approaches** in clinical trial design for both internal and external audiences, through training	Increased # of **CDER programs** that support trial innovation	**Positive impact** on the **drug development landscape** (drug development programs, impact of strategies on trial outcomes)
	Increased **C3TI brand awareness** for both external and internal parties	

To evaluate these outcomes, we plan to review relevant literature, conduct internal and external surveys and interviews, and summarize observations and themes about C3TI activities.

Connect with C3TI!

EMAIL

CDERclinicaltrialinnovation@fda.hhs.gov

WEBSITE

www.fda.gov/C3TI