U.S. FOOD & DRUG ADMINISTRATION

# Complex Innovative Trial Designs

Center for Biologics Evaluation & Research
Center for Drug Evaluation & Research

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# Complex Innovative Trial Designs

The landscape of drug development is evolving, and FDA and industry are facing unique challenges and opportunities.

To modernize drug development, improve efficiency, and promote innovation, the U.S. Food and Drug Administration (FDA) has initiated efforts focused on advancing complex innovative trial designs (CID), which may provide potential benefit across a range of therapeutic areas.

Designs under the CID umbrella include, but are not limited to, complex adaptive, Bayesian, and other novel clinical trial designs which often require simulations to determine the statistical properties of the trial.

## Examples of Complex Innovative Trial Design Features

- Innovative use of external data
- Formal incorporation of prior knowledge
- Inclusion of pre-specified adaptations to multiple aspects of a trial

"One of the most promising ways to make drug development more efficient—while enabling providers and patients to get better information about how a new medicine works—is by developing the science around innovative approaches to the design of clinical trials."

**Scott Gottlieb, M.D.**
Former FDA Commissioner

# The CID Pilot Meeting Program

As part of ongoing CID efforts, FDA launched the Pilot Meeting Program on August 30, 2018. Led by FDA statisticians with participation from relevant disciplines across the agency, the program provides an opportunity for sponsors to interact with experts from FDA at two meetings designed specifically to discuss their proposed CID. The Pilot Meeting Program accepts submission until June 30, 2022.

## Program Eligibility Criteria

The criteria for eligibility include:

- Sponsor has a pre-Investigational New Drug (IND) or IND number
- Proposed CID is intended to provide substantial evidence of effectiveness to support regulatory approval
- There is sufficient clinical information to inform the CID (not a first-in-human study)
- FDA and Sponsor reach an agreement on the trial design information to be publicly disclosed

## Disclosure to Facilitate Learning

CIDs accepted into the pilot will serve as educational resources to facilitate the science and adoption of CIDs. Subject to a disclosure agreement, FDA may present elements of the trial designs as case studies before regulatory approval of the medical product.

# FDA Selection

FDA aims to select a variety of design approaches and therapeutic areas into the CID pilot program. An FDA review group will select up to two CID meeting requests quarterly. Within 45 days after each quarterly closing date, FDA will notify sponsors whether they have advanced to disclosure discussions or their meeting request is denied.

## FDA Evaluation of CID Meeting Requests

- Therapeutic need
- Trial design appropriateness
- Need for simulations
- Level of innovation of the trial design
- Value proposition of the CID

## CID Pilot Meeting Program Benefits

- Innovates medical product development
- Increases dialogue and education among stakeholders
- Advances the use of CIDs
- Develops therapeutic options of benefit to patients

# CID Pilot Meeting Program Quarterly Meeting Request Submission Deadlines

**March 31**

**Sept. 30**

**June 30**

**Dec. 31**

Meeting requests:

- May be submitted on a rolling basis
- Must be received by the last calendar day of each quarter
- Will be received through June 30, 2022

## How to Get More Information

Further information about the CID Pilot Program and submitting a CID meeting request package is available on the CID website: www.fda.gov/CIDpilot.

For specific questions about the CID Pilot Program, please email CID@fda.hhs.gov.