

Advancing Efficient and Inclusive Clinical Trials

Meeting of the Directors of the NCI-designated Cancer
Centers

October 4, 2021

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Director, Center for Drug Evaluation and Research, FDA

FDA Multipronged Approach to Facilitate Innovative, Efficient, and Inclusive Clinical Trials



- FDA Real-World Evidence Program
- Efficient Safety Reporting
- Increasing Diversity in Clinical Trials
- Decentralized Clinical Trials
- Digital Health Tools
- Lessons from the Pandemic

FDA Real-World Evidence Program



FRAMEWORK FOR FDA REAL-WORLD EVIDENCE PROGRAM

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FDA Approves New Use of Transplant Drug Based on Real-World Evidence

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Today, the U.S. Food and Drug Administration approved a new use for [Prograf \(tacrolimus\)](#) based on a non-interventional (observational) study providing [real-world evidence \(RWE\)](#) of effectiveness. FDA approved Prograf for use in combination with other immunosuppressant drugs to prevent organ rejection in adult and pediatric patients receiving lung transplantation.

Content current as of: 07/16/2021

Regulated Product(s): Drugs

Drug Safety and Availability

Information about Nitrosamine Impurities in Medications

We want to provide the clarity needed to realize the full potential of Real-World Data

Real-World Data: Assessing Electronic Health Records and Medical Claims Data To Support Regulatory Decision-Making for Drug and Biological Products

Guidance for Industry

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to <https://www.regulations.gov>. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document or the Real-World Evidence Program, please email CDERMedicalPolicy-RealWorldEvidence@fda.hhs.gov

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Oncology Center of Excellence (OCE)

September 2021
Real World Data/Real World Evidence (RWD/RWE)

2021

2018

We are improving IND safety reporting through guidance and outreach:

- **[Sponsor Responsibilities IND Safety Draft Guidance](#) (June 2021):** Clarifies Sponsor’s IND safety reporting requirements. Expands recommendations for aggregate reporting and safety assessment committees.
 - External [Webinar](#) provided to stakeholders
- **[Investigator Responsibilities for IND Safety Draft Guidance](#) (September 2021):** Focused investigator guidance in this space, including reporting to IRB.
 - External webinar will be provided to stakeholders
- **[WCG Webinar “A Fresh Perspective from the FDA on the Final IND Safety Reporting Rule”](#) (June 2021):** Dr. Temple and Dr. Corrigan-Curay presented regarding sponsor responsibilities for safety reporting.
- **[PRIM&R Annual Conference](#) (Nov 2021):** Collaborative presentation with OGCP highlighting IND safety responsibilities for sponsors, investigators and IRBs.

Inclusion and Diversity in Clinical Trials

- **We are exploring ways to encourage the participation of racial and ethnic minorities and other underrepresented populations in clinical trials through:**
 - **Issuing guidance**
 - *Enhancing the Diversity of Clinical Trial Populations – Eligibility Criteria, Enrollment Practices, and Trial Designs (2020)*
 - *Cancer Clinical Trial Eligibility Criteria: Minimum Age Considerations for Inclusion of Pediatric Patients (2020)*
 - *Inclusion of Older Adults in Cancer Clinical Trials (2020)*
 - *Collection of Race and Ethnicity Data in Clinical Trials (2016)*
 - **Developing tools**
 - CDER developed Drug Trials Snapshots (<https://www.fda.gov/drugs/drug-approvals-and-databases/drug-trials-snapshots>) as part of an overall FDA effort to make demographic data more available and transparent.
 - **Encouraging the use of innovative trial design**
 - FDA is working on guidance pertaining to the use of decentralized trials and digital health technologies.

Facilitating and Encouraging Innovation

e.g., Decentralized Clinical Trials (DCTs)

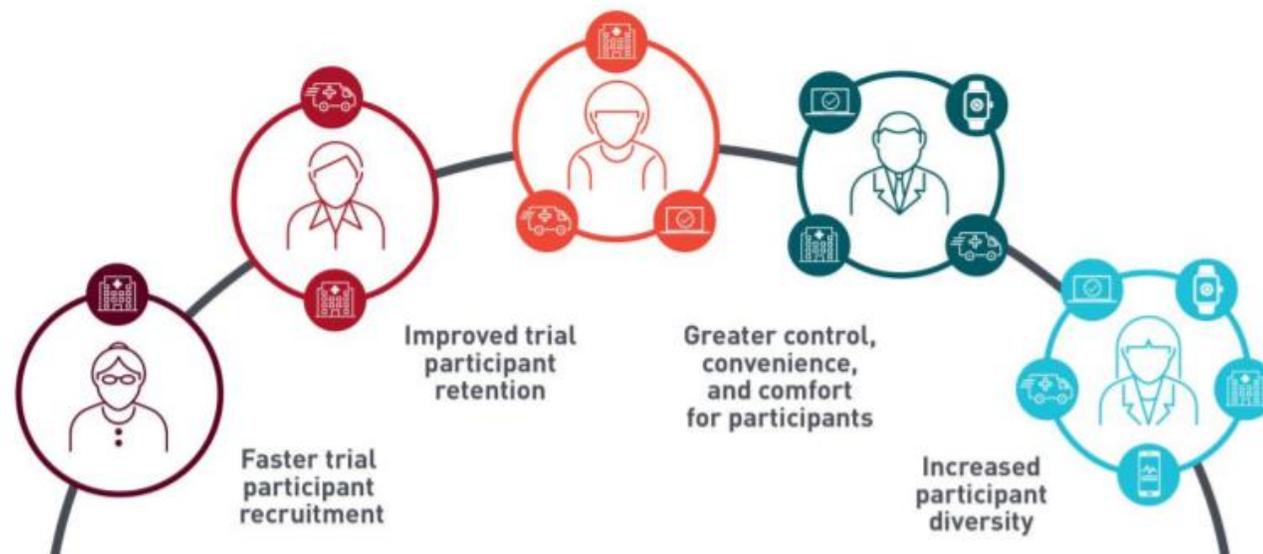


A clinical trial where some or all the trial-related activities occur at a location separate from the investigator's location

Potential benefits:

- Patient convenience (avoiding travel to sites, time off work, etc.)
- Improved inclusivity (patients with mobility, cognitive and economic challenges)
- Ability to study patients in widespread locations (rare or sporadic diseases)

Potential Benefits of Using Decentralized Clinical Trials



FDA Will Issue a Guidance on DCTs

Facilitating and Encouraging Innovation

e.g., Digital Health Tools (DHTs)



A digital health technology (DHT) is a system that uses computing platforms, connectivity, software, and/or sensors, for healthcare and related uses

Potential benefits:

- Ability to study diseases in new ways
- Improved recruitment of patients (e.g., those with limited mobility)
- Data capture outside of health care setting
- Continuous data rather than snapshots
- Objective measurements
- Reduced missing data
- Capturing rare events



FDA Will Issue a Guidance on Digital Health Technologies for Remote Data Acquisition in Clinical Investigations



What Can We Learn From the Pandemic?

FDA

Contains Nonbinding Recommendations

Conduct of Clinical Trials of Medical Products During the COVID-19 Public Health Emergency

Guidance for Industry, Investigators, and Institutional Review Boards

March 2020

Updated on January 27, 2021

For questions on clinical trial conduct during the COVID-19 pandemic, please email
Clinicaltrialconduct-COVID19@fda.hhs.gov.

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Center for Drug Evaluation and Research (CDER)
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Office of Good Clinical Practice (OGCP)

- Initial release date: March 18, 2020
- Most recent update: August 30, 2021

Lessons and examples from public health emergencies:

- Leveraging existing healthcare infrastructure
- Remote assessment and monitoring
- Identifying & prioritizing critical processes
- Leveraging technology and innovations
- Effective communication and documentation



Summary

- We recognize the great potential in many evolving areas to advance drug development.
- We are working to provide the needed regulatory perspective to not only facilitate, but also encourage innovations.
- We are developing multiple guidances to explore the utility of RWD & RWE, and to facilitate a more efficient clinical trial design and conduct.
- We are engaging with multiple partners across the Federal Government and the private sector to disseminate knowledge and to help facilitate innovative approaches.