Regulatory Perspectives: Development and Qualification of Novel Drug Development Tools

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21st Century Cures Act

- Signed into law: December 13, 2016

- Adds new section 507 to the Food, Drug, and Cosmetic Act (FD&C Act) concerning the qualification of DDTs
  - Subtitle B—Advancing New Drug Therapies
  - Sec. 3011. Qualification of drug development tools

- Legislation establishes new processes for qualification of DDTs (biomarkers and clinical outcome assessments)

https://www.congress.gov/bill/114th-congress/house-bill/34/text
https://www.fda.gov/media/155003/download?attachment

21st CC DDT Process (Section 3011)

- Formalizes a process defined by three submissions. “Accept” or “Not Accept” decision for each:
  - Letter of Intent (LOI)
  - Qualification Plan (QP)
  - Full Qualification Package (FQP)
- Requires setting and implementing “reasonable timeframes” for the FDA review of each submission type

Review and Decision Process

- Each DDT Program Assessment and Reviewability
  - Does the Submission contain the needed information to undergo a full review.
- Qualification Review Team
  - Discipline-specific SME Assessment and Recommendations
  - Includes OND division management participation
  - Evaluate based on regulatory precedent, current disease-specific challenges, and level of impact on drug development programs
- CDER/CBER DDT Committee
  - Opportunity for broad senior CDER input early and throughout in the process
  - Vote on Decision to Accept or Reject submission at each stage

Qualification Process for Drug Development Tools

Guidance for Industry and FDA Staff

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)

November 2020
Drug Development Tools

Lessons Learned

How to Engage

FDA Patient Listening Sessions

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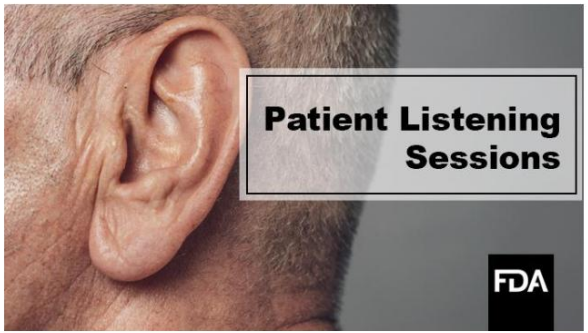

FDA Patient Affairs hosts Patient Listening Sessions. These sessions are a resource for the medical product Centers to engage with patients and their advocates. Patient Listening Sessions are one of many ways the patient and advocacy community can share their experiences and perspectives by talking directly with FDA staff.

Upcoming scheduled Patient Listening Session topics:

https://www.fda.gov/patients/learn-about-fda-patient-engagement/fda-patient-listening-sessions

CDER Patient-Focused Drug Development

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What is Patient-Focused Drug Development?

Patient-focused drug development (PFDD) is a systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. As experts in what it is like to live with their condition, patients are uniquely positioned to inform the understanding of the therapeutic context for drug development and evaluation.

The primary goal of patient-focused drug development is to better incorporate the patient's voice in drug development and evaluation, including but not limited to:

- Facilitating and advancing use of systematic approaches to collecting and utilizing robust and meaningful patient and caregiver input to more consistently inform drug

https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

Critical Path Innovation Meetings (CPIM)

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Watch a video about the CPIM program

The Critical Path Innovation Meeting (CPIM) was developed by CDER to address issues in drug development identified in the 2004 FDA publication, *Innovation or Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products Report.* The report identified several areas of product development in need of improvement, including "technical methods such as animal or computer-based predictive models, biomarkers for safety and effectiveness, and new clinical evaluation techniques," and cited a need "to create better tools for developing medical technologies [and] a knowledge base built not just on ideas from biomedical research, but on reliable insights into the pathway to patients."

The CPIM is a means by which the Center for Drug Evaluation and Research (CDER) and investigators from industry, academia, scientific consortia, patient advocacy groups, and government can communicate to improve efficiency and success in drug development. The goals of the CPIM are to discuss a methodology or technology proposed by the meeting requester and for CDER to provide general advice on how this methodology or technology might enhance drug development. CDER will identify some of the larger gaps in existing knowledge that requesters might consider addressing during their work. CDER expects to become more familiar with prospective innovations in drug development, broadening its regulatory perspective. CPIM discussions are non-regulatory, drug product-independent and nonbinding on both FDA and CPIM requesters.

The CPIM is a forum for FDA and stakeholders to discuss potential scientific

https://www.fda.gov/drugs/novel-drug-approvals-fda/critical-path-innovation-meetings-cpim

How to Engage

Drug Development Tool (DDT) Qualification Programs

Spotlight Events & Announcements

Biomarker Qualification Program - Publication of Revised Qualification Plan Content Element Outline

The FDA has published a revised version of the Biomarker Qualification Program (BQP) Qualification Plan Content Element Outline (July 2025). This updated document provides requestors with comprehensive instructions for preparing Qualification Plan submissions under the Drug Development Tool (DDT) qualification process established by Section 507 of the 21st Century Cures Act. You may access the document from the Resources for Biomarker Requestors website: https://www.fda.gov/drugs/biomarker-qualification-program/resources-biomarker-requestors.

To locate a project or a qualified biomarker go to CDER & CBER's DDT Qualification

https://www.fda.gov/drugs/development-approval-process-drugs/drug-development-tool-ddt-qualification-programs

Digital Health Technologies (DHTs) for Drug Development

Digital health technologies (DHTs) offer many potential benefits in the development of medical products, including drugs. Advances in DHTs, including electronic sensors, computing platforms and information technology, provide new opportunities to obtain clinical trial data directly from patients. Portable DHTs that may be worn, implanted, ingested, or placed in the environment allow real-time collection of data from trial participants in their homes or at locations remote from clinical trial sites. Potential advantages of these DHTs include the ability to:

- make continuous or frequent measurements of clinical features
- record or measure novel clinical features that could not be captured during traditional study visits
- decentralize clinical trial activities by obtaining clinical data from study participants remotely

FDA is committed to supporting the use of DHTs in clinical drug development and has developed a comprehensive program to engage with interested parties in this important scientific area.

The Prescription Drug User Fee Act VII has outlined several activities related to DHTs for drug development and review, which FDA has committed to undertake. These activities include:

https://www.fda.gov/science-research/science-and-research-special-topics/digital-health-technologies-dhts-drug-development


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