FDA Drug Development Resources for the Rare Disease Community and More

FDA Drug Development Websites

Investigational New Drug (IND) Application Information
https://www.fda.gov/drugs/types-applications/investigational-new-drug-ind-application

IND Applications for Clinical Investigations: Regulatory and Administrative Components
IND Applications for Clinical Investigations: Regulatory and Administrative Components | FDA

Requesting a Pre-Assigned Application number (e.g., IND, NDA)
https://www.fda.gov/drugs/electronic-regulatory-submission-and-review/requesting-pre-assigned-application-number

IND Forms and Instructions
https://www.fda.gov/drugs/investigational-new-drug-ind-application/ind-forms-and-instructions

CDER Offices and Divisions (use to identify a project manager in an Office of New Drugs division for a particular disease)
https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/cder-offices-and-divisions

Investigator Initiated IND
https://www.fda.gov/drugs/investigational-new-drug-ind-application/investigator-initiated-investigational-new-drug-ind-applications

CDER Small Business & Industry Assistance (SBIA): A Comprehensive Resource for Information on Human Drug Development in Regulation
https://www.fda.gov/drugs/development-approval-process-drugs/cder-small-business-industry-assistance-sbia

Biomarkers
https://www.fda.gov/drugs/biomarker-qualification-program/about-biomarkers-and-qualification

Complex Innovative Trial Design (CID)
https://www.fda.gov/drugs/development-resources/complex-innovative-trial-design-meeting-program

Model-Informed Drug Development (MIDD)
https://www.fda.gov/drugs/development-resources/model-informed-drug-development-pilot-program

Advancing Oncology Decentralized Trials: Learning from COVID-19 Trial Datasets
https://www.fda.gov/about-fda/oncology-center-excellence/advancing-oncology-decentralized-trials

Critical Path Innovation Meetings (CPIM)
https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/critical-path-innovation-meetings-cpim

CDER Patient-Focused Drug Development (PFDD)
https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

Updated May 11, 2022

IND Regulations

IND Application - 21 CFR Part 312.20
https://www.ecfr.gov/current/title-21/chapter-I/subchapter-D/part-312

- 21 CFR 312.23 content and format

See the IND Application website for additional IND-related regulations
https://www.fda.gov/drugs/types-applications/investigational-new-drug-ind-application

Drug Development Guidances

Landing page for document search:
Search for FDA Guidance Documents | FDA

Substantial Evidence of Effectiveness – Draft Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/demonstrating-substantial-evidence-effectiveness-human-drug-and-biological-products

Benefit-Risk Assessment for New Drug and Biological Products – Draft Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/benefit-risk-assessment-new-drug-and-biological-products

Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products Guidance for Industry – Draft Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/formal-meetings-between-fda-and-sponsors-or-applicants-pdufa-products-guidance-industry

Adaptive Designs for Clinical Trials of Drugs and Biologics – Final Guidance
https://www.fda.gov/media/78495/download

Rare Diseases: Early Drug Development and the Role of Pre-IND Meetings – Draft Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/rare-diseases-early-drug-development-and-role-pre-ind-meetings

Investigational New Drug Applications (INDs) — Determining Whether Human Research Studies Can Be Conducted Without an IND - Final Guidance
https://www.fda.gov/media/79386/download

Considerations for the Use of Real-World Data and Real-World Evidence To Support Regulatory Decision-Making for Drug and Biological Products – Draft Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/considerations-use-real-world-data-and-real-world-evidence-support-regulatory-decision-making-drug

Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products – Draft Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/real-world-data-assessing-registries-support-regulatory-decision-making-drug-and-biological-products

Updated May 11, 2022

Digital Health Technologies for Remote Data Acquisition in Clinical Investigations – Draft Guidance
https://www.fda.gov/media/155022/download

Rare Diseases: Common Issues in Drug Development – Draft Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/rare-diseases-common-issues-drug-development-guidance-industry

Rare Diseases: Natural History Studies for Drug Development – Draft Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/rare-diseases-natural-history-studies-drug-development

Qualification Process for Drug Development Tools – Final Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/qualification-process-drug-development-tools-guidance-industry-and-fda-staff

Botanical Drug Development – Final Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/botanical-drug-development-guidance-industry

Pediatric Rare Diseases--A Collaborative Approach for Drug Development Using Gaucher Disease as a Model – Draft Guidance
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/pediatric-rare-diseases-collaborative-approach-drug-development-using-gaucher-disease-model-draft

International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) Guidelines

Efficacy Guidelines
https://www.ich.org/page/efficacy-guidelines

See:
E6 - Good Clinical Practice Guidelines
E9 – Statistical Principles for Clinical Trials

Safety Guidelines
https://www.ich.org/page/safety-guidelines

See:
S1A - Need for Carcinogenicity Studies of Pharmaceuticals
S1B – Testing for Carcinogenicity of Pharmaceuticals
S1C – Dose Selection for Carcinogenicity Studies of Pharmaceuticals

Educational Resources

CDERLearn Training and Education
https://www.fda.gov/training-and-continuing-education/cderlearn-training-and-education

Clinical Investigator Training Course (CITC)
Clinical Investigator Training Course (CITC) Update - 12/07/2021 - 12/08/2021 | FDA

Updated May 11, 2022