Regulatory Resources and Avenues for Obtaining Early Guidance from CBER/OTP

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FDA | NIH: Regulatory Do's and Don'ts: Tips from FDA
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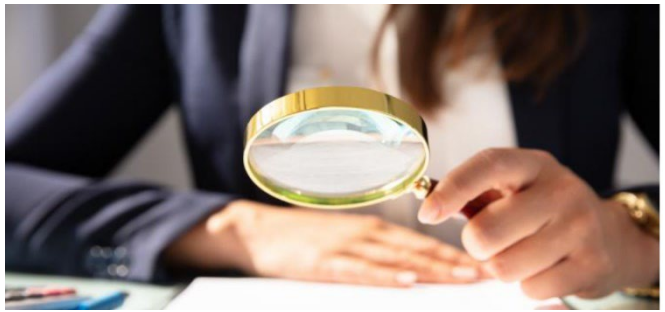
Learning Objectives

- Locate resources for interacting with CBER's Office of Therapeutic Products (OTP)
- Understand communication, submission, and meeting options for early interaction with OTP
- Enhance your understanding of processes through OTP Learn
- Recognize additional regulatory resources for your future efforts



Who is CBER/OTP?

- CBER OTP is one of three CBER product offices
- OTP oversees development of biological products, including:
  - Purified and recombinant proteins for hematology
  - Antivenins
  - Gene and cell therapies
  - Therapeutic tissue engineered products
  - Human tissue products
  - Therapeutic vaccines and antigen-specific active immunotherapies
  - Certain medical devices
  - Xenotransplantation products

Interactions with OTP

- Multiple opportunities to interact with OTP
- Submissions sent to CBER's Document Control Center, per: Regulatory Submissions in Electronic and Paper Format for CBER-Regulated Products | FDA

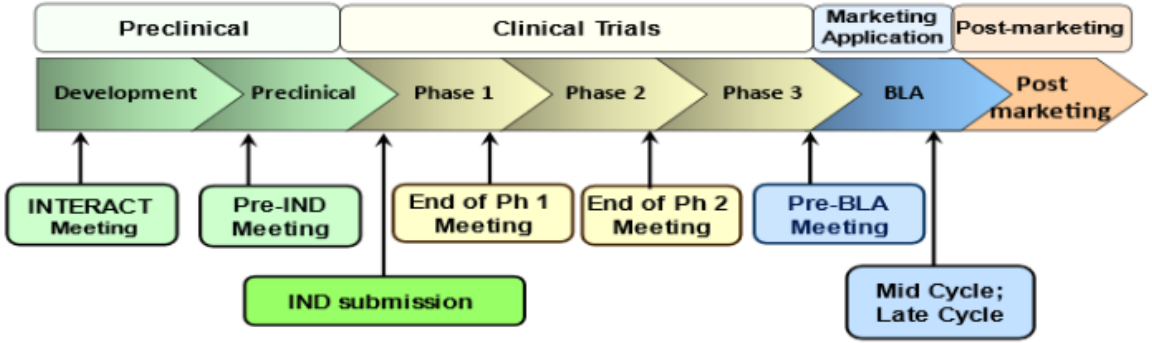
  –Secure email policy: CBER SOPP 8119: Use of Email for Regulatory Communications
- Assigned Regulatory Project Manager (RPM) is the point of contact
- Scheduling, conducting, and documenting formal meetings detailed in FDA regulations, guidance documents, and SOPs
- Comprehensive set of OTP webpages to guide Sponsors: Interactions with Office of Therapeutic Products | FDA

Interactions with OTP (cont.)

- Scope: Website (Interactions with Office of Therapeutic Products | FDA) and presentation to address biological products regulated under IND/BLA pathway
- OTP regulated medical devices, refer to Requests for Feedback and Meetings for Medical Device Submissions: The Q-Submission Program: Guidance for Industry and Food and Drug Administration Staff

Formal Meetings - Overview

- Include Meeting Types A, B, C, D, INTERACT and requests for clarification
- Meeting formats:
  - Face-to-face (in-person and virtual meetings on IT platforms)
  - Teleconference
  - Written Response Only (WRO)
- Most formal meetings available throughout product lifecycle



INTERACT Meetings

- **IN**itial **T**argeted **E**ngagement for **R**egulatory **A**dvice on **C**BER/CDER Produc**T**s meetings
- Sponsors developing novel therapies
- Feedback at an early stage. Should have:
  - Identified investigational product
  - Conducted preliminary preclinical proof-of-concept studies
  - Not conducted definitive toxicology studies
- Webpage*: Procedures, meeting request/package content, timelines

* Interactions with Office of Therapeutic Products | FDA

Pre-IND Meetings

- Type B, Pre-IND Meeting; Sponsor may obtain guidance on the following (examples):
  - Target product profile
  - Design of preclinical studies and initial IND study
  - Product manufacturing & quality controls; to initiate human studies
  - Pediatric population considerations
  - Best approach for presenting the data in the IND
- Assist sponsors to prepare an IND application; reduce risk of a clinical hold
- Webpage*: Procedures, meeting request/package content, timelines

* Interactions with Office of Therapeutic Products | FDA

Additional Formal Meetings

- Different categories of Meetings:
  - Type A – Stalled program
  - Type B - End of Phase; Pre-BLA; BT & RMAT
  - Type C – Request is not Type A or Type B Meeting
  - Type D – Limited number of topics, questions and disciplines
- Request for Clarifications
  - Sought after a Type A, B, C, D and INTERACT meeting
- Webpage*: Procedures, meeting request/package content, timelines

* Interactions with Office of Therapeutic Products | FDA

Additional Interactions

- CBER Advanced Technologies Team (CATT) meeting
  - Prospective innovators and developers of advanced manufacturing and testing technologies
  - Discuss novel technology rather than a specific therapeutic product.

OTP Regulatory Resources

- **OTP Learn:** OTP Learn | FDA
- Currently provides 30 webinars; examples:
  - Original IND content and format
  - IND Decisions: Safe to Proceed, Clinical Hold and Partial Hold
  - CMC Section of a Gene Therapy IND
  - RMAT and BT designation

OTP Regulatory Resources (cont.)

- OTP Events, Meetings, and Workshops | FDA
- Includes 22 recordings
- **OTP Town Hall** series:
  - Engage product developers/ researchers
  - Discuss topics related to OTP-regulated products; Q&A format
  - Recent topics include ‘Nonclinical Assessment of Cell and Gene Therapy Products’, and ‘Cell Therapy Chemistry Manufacturing and Controls’

OTP Regulatory Resources (cont.)

- OTP Events, Meetings, and Workshops | FDA (cont.)
- **RegenMedEd**; series on Regenerative Medicine
  - Engage stakeholders; discuss regenerative medicine therapies
  - Explore opportunities for patients, caregivers, and advocates to engage with OTP, to help advance product development.
  - Recent topics include ‘Clinical Trials: The Patient Experience’ and ‘Regenerative Medicine 101’

OTP Regulatory Resources (cont.)

- Cellular & Gene Therapy Guidances | FDA
  - Outlines 36 Guidances for Industry, and in some cases a link to a related webinar

- For general regulatory support and procedural questions, please feel free to contact OTPRPMS@fda.hhs.gov

OTP Resource Weblinks

- Overarching Meeting Resources:
  - Interactions with Office of Therapeutic Products | FDA, containing additional links within the document
  - Formal Meetings Between the FDA & Sponsors or Applicants of PDUFA Products Guidance for Industry
  - SOPP 8101.1: Regulatory Meetings with Sponsors and Applicants for Drugs and Biological Products

OTP Resource Weblinks (cont.)

- Guidance Documents supporting product development:
  - Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (INDs)
  - Guidance for FDA Reviewers and Sponsors: Content and Review of Chemistry, Manufacturing, and Control (CMC) Information for Human Somatic Cell Therapy Investigational New Drug Applications (INDs)
  - Guidance for Industry: Preclinical Assessment of Investigational Cellular and Gene Therapy Products
  - Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products; Guidance for Industry

OTP Resource Weblinks (cont.)

- Additional Guidance documents: Cellular & Gene Therapy Guidances | FDA
- Training, webinars and workshops:
  - OTP Learn | FDA
  - OTP Events, Meetings, and Workshops | FDA
- Questions for OTP? Email: OTPRPMS@fda.hhs.gov
- Resources for sending applications/ submissions to CBER: Regulatory Submissions in Electronic and Paper Format for CBER-Regulated Products | FDA
- Secure email policy: CBER SOPP 8119: Use of Email for Regulatory Communications

Summary

- Many opportunities for interaction with OTP, throughout the product life cycle
- Early engagement recommended
- Numerous online resources to:
  - Enhance understanding
  - Facilitate interactions with OTP

Contact Information

U.S. FOOD & DRUG ADMINISTRATION

- **Regulatory Questions:**
  OTP Main Line – 240.402.8190
  Email: OTPRPMS@fda.hhs.gov
- **Interactions with Office of Therapeutic Products website:**
  Interactions with Office of Therapeutic Products | FDA
- **OTP Learn Webinar Series:**
  http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/ucm232821.htm

- **CBER website:** www.fda.gov/BiologicsBloodVaccines/default.htm
- **Phone: 1-800-835-4709 or 240-402-8010**
- **Consumer Affairs Branch:** ocod@fda.hhs.gov
- **Manufacturers Assistance and Technical Training Branch:**
  industry.biologics@fda.hhs.gov
- **Follow us on X, formerly Twitter:** https://www.twitter.com/fdacber