

# 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.)

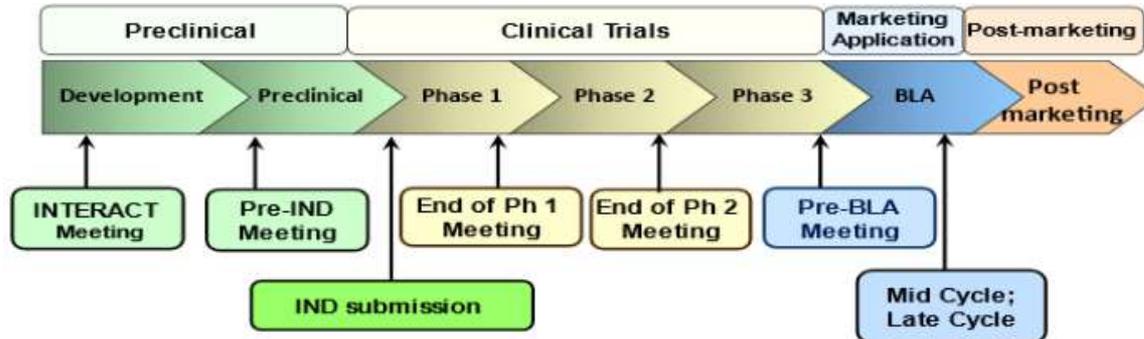


- Scope: Website ([Interactions with Office of Therapeutic Products | FDA](#)) and presentation to address biological products regulated under IND/BLA pathway
- OTP regulated medical devices, refer to [Requests for Feedback and Meetings for Medical Device Submissions: The Q-Submission Program: Guidance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



- **Initial Targeted Engagement for Regulatory Advice on CBER/CDER Products** 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](mailto: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](mailto: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](mailto: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](http://www.fda.gov/BiologicsBloodVaccines/default.htm)
- **Phone:** 1-800-835-4709 or 240-402-8010
- **Consumer Affairs Branch:** [ocod@fda.hhs.gov](mailto:ocod@fda.hhs.gov)
- **Manufacturers Assistance and Technical Training Branch:**  
[industry.biologics@fda.hhs.gov](mailto:industry.biologics@fda.hhs.gov)
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