FDA/MCERSI Hybrid Workshop:
Clinical Pharmacology Guidances Advancing Drug Development and Regulatory Assessment: Role and Opportunities

Date and Time:
May 8 and 9, 2024
9:00am-2:30pm (ET)

Location:
Building 31, FDA Great Room
FDA White Oak Campus
10903 New Hampshire Avenue
Silver Spring, MD 20903,
Zoom

About This Event:
This public workshop will be hosted by the FDA Office of Clinical Pharmacology (OCP) and the University of Maryland Center of Excellence in Regulatory Science and Innovation (M-CERSI). The purpose of this workshop is to serve as a mechanism to engage with the public to share information on existing clinical pharmacology guidances and identify emerging scientific topics that might benefit from further scientific research and recommendations.

Background:
At every step of drug development, clinical pharmacology is applied to generate, evaluate, and use knowledge of drug disposition, pharmacology, and disease biology to progressively reduce regulatory uncertainty and inform decision-making. Clinical pharmacology principles form the basis of dosage selection and optimization and promote therapeutic individualization by translating the knowledge of patient diversity into clinical recommendations for safe and effective use of medications. Therefore, having clear, pragmatic, and contemporary scientific recommendations to inform drug development and regulatory assessment is critical for the successful and efficient development of therapeutics that protect and promote public health. Developing new or revising existing guidance documents, especially in the broad field of clinical pharmacology, requires a unique and integrated approach centered around multistakeholder partnerships.

Workshop Objectives:
1. Provide an overview of scientific recommendations pertaining to clinical pharmacology applications during drug development and regulatory assessment.
2. Discuss the current scientific challenges and gaps in applying clinical pharmacology principles during drug development.
3. Identify potential opportunities and priorities for regulatory research and scientific guidance development from a clinical pharmacology perspective.
**FDA/MCERSI Hybrid Workshop:**

**Clinical Pharmacology Guidances Advancing Drug Development and Regulatory Assessment: Role and Opportunities**

**Agenda**

**Day 1 – Wednesday, May 8, 2024, 9:00am-2:00pm (ET)**

### Session 1 – Opening & Overview

**Session Objectives:**
- Provide context for the workshop
- Discuss the importance of guidances in advancing drug development and regulatory decision-making
- Discuss the role of regulatory research in informing guidance development

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<tr>
<th>Time</th>
<th>Activity</th>
<th>Speaker/Institution</th>
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<tbody>
<tr>
<td>9:00am-9:10am</td>
<td>Welcome and opening remarks</td>
<td>Issam Zineh, OCP, FDA</td>
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<tr>
<td>9:10am-9:25am</td>
<td>Role of regulatory science in informing regulatory decision making</td>
<td>Peter Stein, OND, FDA</td>
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<td>9:25am-9:40am</td>
<td>Academic research informing FDA evidence based guidances</td>
<td>Kathy Giacomini, UCSF</td>
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<td>9:40am-9:55am</td>
<td>Advancing clinical pharmacology innovation through regulatory policy: an industry perspective</td>
<td>Michelle Rohrer, Genentech/Roche</td>
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<td>9:55am-10:00am</td>
<td>Break</td>
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### Session 2 – Clinical pharmacology considerations for specific patient subpopulations

**Session Objectives:**
- Discuss the clinical pharmacology considerations for specific patient populations (e.g., older adults, obese patients, pregnant or lactating persons, sex, race, and ethnicity) during drug development
- Understand scientific gaps and identify future opportunities

**Moderator:** Anu Ramamoorthy, OCP, FDA

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<tr>
<th>Time</th>
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<tbody>
<tr>
<td>10:00am-10:20am</td>
<td>Therapeutic individualization - Clinical pharmacology in addressing specific populations</td>
<td>Elimika Pfuma Fletcher, OCP, FDA</td>
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<td>10:20am-10:40am</td>
<td>Clinical Pharmacology approaches to support subgroup analyses during drug development</td>
<td>Aarti Sawant, IQ, AstraZeneca</td>
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<td>10:40am-11:00am</td>
<td>Drug development considerations for pregnant and lactating individuals Panel Discussion: Opportunities and challenges in applying clinical pharmacology principles to inform use of drugs in specific populations</td>
<td>Sara Quinney, Indiana University School of Medicine All Speakers + Michael Neely, Children’s Hospital Los Angeles</td>
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<td>11:00am-11:30am</td>
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<td>11:30am-12:30pm</td>
<td>Lunch</td>
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### Session 3 - Application of clinical pharmacology for rare disease drug development

**Session Objectives:**
1. Discuss the expectations in implementing clinical pharmacology principles and guidances during the development of drugs for rare diseases
2. Understand challenges in implementation and identify future opportunities

**Moderator:** Michael Pacanowski, OCP, FDA

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<tr>
<th>Time</th>
<th>Topic</th>
<th>Presenter</th>
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<tbody>
<tr>
<td>12:30pm–12:50pm</td>
<td>Clinical pharmacology in drug development for rare diseases</td>
<td>Robert Schuck, OCP, FDA</td>
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<tr>
<td>12:50pm–1:10pm</td>
<td>Practical considerations for conducting clinical pharmacology studies during drug development for rare diseases</td>
<td>Marshall Summar, Uncommon Cures</td>
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<tr>
<td>1:10pm–1:30pm</td>
<td>Integrated applied clinical pharmacology in the advancement of rare/ultra-rare disease therapeutics</td>
<td>Steven Ryder, Rallybio</td>
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<tr>
<td>1:30pm–2:00pm</td>
<td>Panel Discussion: Challenges in drug development for rare diseases and science and policy opportunities</td>
<td>All Speakers</td>
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### Day 2 – Thursday, May 9, 2024, 9:00am–2:30pm (ET)

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<th>Time</th>
<th>Session</th>
<th>Presenter</th>
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<tr>
<td>9:00am–9:10am</td>
<td>Welcome and Recap of Day 1</td>
<td>Raj Madabushi, OCP, FDA</td>
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### Session 4 – Global harmonization of clinical pharmacology considerations

**Session Objectives:**
1. Discuss how drug development could benefit from global harmonization of clinical pharmacology principles and concepts

**Moderator:** Kellie Reynolds, OCP, FDA

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<th>Time</th>
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<tr>
<td>9:10am–9:25am</td>
<td>Global regulatory harmonization</td>
<td>Xinning Yang, OCP, FDA</td>
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<td>9:25am–9:40am</td>
<td>Clinical pharmacology guidances advancing drug development and regulatory assessment: an industry perspective</td>
<td>Vikram Sinha, Novartis</td>
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<td>9:40am–10:10am</td>
<td>Panel discussion: Current harmonization activities and future harmonization needs</td>
<td>All Speakers + Paulo Paixão, Portuguese National Authority of Medicines and Health Products (INFARMED), Jenny Chien, Eli Lilly and Company, Akihiro Ishiguro, Pharmaceuticals and Medical Devices Agency (PMDA)</td>
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<td>10:10am–10:15am</td>
<td>Break</td>
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Session 5 – Role of quantitative medicine in drug development and decision making

Session Objectives:
1) Provide an overview of clinical pharmacology guidances that provide recommendations on model-based approaches to support drug development and regulatory decision-making
2) Identify gaps and future opportunities

Moderator: Raj Madabushi, OCP, FDA

10:15am–10:35am
Quantitative clinical pharmacology: Tools for new drug and policy development
Hao Zhu, OCP, FDA

10:35am–10:55am
Industry perspective on clinical pharmacology guidances advancing drug development and regulatory assessment
Daniele Ouellet, Johnson & Johnson

10:55am–11:15am
What FDA guidances do we need for clinical pharmacology?
Joga Gobburu, University of Maryland, Baltimore

11:15am–11:45am
Panel Discussion: Challenges faced, potential gaps, and topics that could warrant future potential regulatory research and scientific recommendation
All Speakers + Karen Rowland Yeo, Certara

11:45am–12:30pm
Lunch

Session 6 – Clinical pharmacology guidances supporting the lifecycle of a new therapeutic product

Session Objectives:
1) Discuss clinical pharmacology principles, guidances, and gaps that apply across the lifecycle of a new therapeutic product

Moderator: James Polli, University of Maryland

12:30pm–12:40pm
Overview of clinical pharmacology guidances that apply across the lifecycle of drugs
Ethan Stier, OCP, FDA

12:40pm–1:05pm
Do FDA clinical pharmacology guidances adequately support product development & lifecycle?
Roger Nosal, NGT BioPharma Consultants

1:05pm–1:25pm
Modeling and simulation lifecycle considerations
Xavier Pepin, Simulations Plus

1:25pm–2:00pm
Panel Discussion: Challenges encountered during the lifecycle of new therapeutic products and further opportunities to apply clinical pharmacology principles
All Speakers + Kimberly Raines, OPQ, FDA, Hao Zhu, OCP, FDA

Session 7 – Closing Remarks

Session Objectives:
1) To summarize the learnings from the workshop and outline potential next steps

2:00pm–2:10pm
Summarization of gaps and challenges, and short- and long-term scientific recommendation needs
Anu Ramamoorthy, OCP, FDA

2:10pm–2:20pm
Summarization of gaps and challenges, and short- and long-term regulatory research needs
James Polli, University of Maryland