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.

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Agenda

(*Titles Tentative*)

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

Session 1 – Opening & Overview

Session Objectives:

1) Provide context for the workshop
2) Discuss the importance of guidances in advancing drug development and regulatory decision-making
3) Discuss the role of regulatory research in informing guidance development

9:00am-9:10am	Welcome and Opening Remarks	Issam Zineh, OCP, FDA
9:10am-9:25am	Role of regulatory science in informing regulatory decision making	Peter Stein, OND, FDA
9:25am-9:40am	Academic research informing FDA evidence based guidances	Kathy Giacomini, UCSF
9:40am-9:55am	Industry perspective	Michelle Rohrer, Genentech/Roche
9:55am-10:00am	Break	

Session 2 - Clinical pharmacology considerations for specific patient subpopulations

Session Objectives:

1) 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
2) Understand scientific gaps and identify future opportunities for regulatory research and scientific recommendation development

Moderator: Anu Ramamoorthy, OCP, FDA

10:00am-10:20am	Therapeutic individualization - Clinical pharmacology in addressing specific populations	Elimika Pfuma Fletcher, OCP, FDA
10:20am-10:40am	Clinical Pharmacology approaches to support subgroup analyses during drug development	Aarti Sawant, IQ, AstraZeneca
10:40am-11:00am	Drug development considerations for pregnant and lactating individuals	Sara Quinney, Indiana University School of Medicine
11:00am-11:30am	**Panel Discussion:** Challenges in studying specific patient populations and leveraging clinical pharmacology principles to extrapolate to unstudied or understudied populations	All Speakers + Michael Neely, Children's Hospital Los Angeles
11:30am-12:30pm	Lunch	

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

12:30pm-12:50pm	Application of clinical pharmacology guidances during drug development for rare diseases: timing and design of essential studies	Robert Schuck, OCP, FDA
12:50pm-1:10pm	Practical considerations for conducting clinical pharmacology studies during drug development for rare diseases	Marshall Summar, Uncommon Cures
1:10pm-1:30pm	Integrated applied clinical pharmacology in the development of rare and ultra-rare disease therapeutics: An overview and case studies	Steven Ryder, Rallybio
1:30pm-2:00pm	**Panel Discussion:** Challenges in drug development for rare diseases and science and policy opportunities	All Speakers

Day 2 – Thursday, May 9, 2024, 9:00am-2:30pm (ET)

9:00am-9:10am	Welcome and Recap of Day 1	Anu Ramamoorthy, OCP, FDA

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

9:10am-9:25am	Global regulatory harmonization	Xinning Yang, OCP, FDA
9:25am-9:40am	Clinical pharmacology guidances advancing drug development and regulatory assessment: An Industry Perspective	Vikram Sinha, Novartis
9:40am-10:10am	**Panel discussion:** Current harmonization activities and future harmonization needs	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)
10:10am-10:15am	Break	

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) Understand challenges in implementation and identify future opportunities

Moderator: Raj Madabushi, OCP, FDA

10:15am-10:35am	Overview of clinical pharmacology guidances providing recommendations on quantitative approaches used during drug development and regulatory assessment	Hao Zhu, OCP, FDA
10:35am-10:55am	Practical applications of clinical pharmacology guidances providing recommendations on quantitative approaches	Daniele Ouellet, J&J
10:55am-11:15am	What FDA guidances do we need for clinical pharmacology?	Joga Gobburu, UMD
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-12:35pm	Overview of clinical pharmacology guidances that apply across the lifecycle of drugs	Ethan Stier, OCP, FDA
12:35pm-12:55pm	Pre-approval lifecycle considerations	Roger Nosal, NGT BioPharma Consultants
12:55pm-1:15pm	Post-approval lifecycle considerations	Sarah Robertson, Roche
1:15pm-1:35pm	Modeling and simulation lifecycle considerations	Xavier Pepin, Simulations Plus
1:35pm-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 guidance needs	Raj Madabushi, OCP, FDA
2:10pm-2:20pm	Summarization of gaps and challenges, and short- and long-term regulatory research needs	James Polli, University of Maryland