Public Stakeholder Meeting on Prescription Drug User Fee Act (PDUFA) Reauthorization

October 30, 2020

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Outline for this meeting

• Welcome and Roll Call
• Presentation Topics:
  • Patient Focused Drug Development
  • Model-Informed Drug Development
  • Complex Innovative Designs for Clinical Trials
  • Other Areas of Regulatory Science: Advancing Translational Models & Tools
• Topics for upcoming meetings
Model Informed Drug Development (MIDD)

October 30, 2020
Advancing Model-Informed Drug Development (MIDD)

Background:
• Development and application of exposure-based, biological, and statistical models derived from preclinical and clinical data sources to address drug development or regulatory issues

• MIDD can improve efficiency in drug development and address residual regulatory uncertainty

• Prior to PDUFA VI, application of MIDD principles were not routinely integrated into drug development
  • Lack of consistent application and uniformity of acceptance
  • Absences of best practices and contemporary guidance to make FDA expectations transparent
  • Unknown resource needs for FDA
MIDD - What was committed to in PDUFA VI?

• FDA will develop its regulatory science and review expertise and capacity in MIDD approaches.

• FDA will convene a series of workshops to identify best practices for MIDD. Topics include physiologically-based pharmacokinetic modeling, design analysis and inferences from dose-exposure-response, disease progression model development, immunogenicity.

• FDA will conduct a pilot program for MIDD approaches. For sponsors participating in the pilot program, FDA will grant a pair of meetings specifically designed for this pilot program. FDA will select 1-2 proposal per Center per quarter.

• FDA will publish draft guidance, or revise relevant existing guidance, on model-informed drug development.

• FDA will develop or revise, as appropriate, relevant MAPPs or SOPPs, and/or review templates and training, to incorporate guidelines for the evaluation of MIDD approaches.

MIDD – PDUFA VI Experience

• Conducted two MIDD Public workshops
  • Design Analysis and Inferences from Dose-Exposure-Response (2018)
  • Physiologically Based Pharmacokinetic Modeling (2019)
  • Disease Progression Model Development – Planned for 2021
  • Immunogenicity – Planned for 2021

• Published two MIDD Guidance Documents
  • Physiologically Based Pharmacokinetic Analysis – Format and Content (De novo; Final – 2018)
  • Population Pharmacokinetics (Revised 1999 guidance; Draft – 2019)
  • SOP for Population Pharmacokinetic Analysis (On track to be established in 2020-21)

• Launched MIDD Paired Meeting Pilot Program
  • Published FRN outlining the eligibility criteria and procedures for submission (2018)
  • Established a cross-center MIDD Selection Committee to review meeting requests (2018)
  • Operationalized the MIDD Paired Meeting Pilot Program (2018)

MIDD Website - https://www.fda.gov/drugs/development-resources/model-informed-drug-development-pilot-program
MIDD Paired Meeting Pilot Program experience

- Received 32 meeting requests (19 different sponsors) over 9 quarters
- Granted 28 meeting requests (~3/quarter)
- Facilitated 2 regulatory submissions
Advancing Model-Informed Drug Development

- Creating an environment that increases stakeholder acceptance of MIDD approaches
- Developing standards and best practices that lead to consistent application and evaluation
- Increasing capacity and expertise to address growing demands and innovation