Disease Progression Modeling to Advance Clinical Trial Decision Making

Multi-Stakeholder Expert Meeting Agenda

March 6, 2023

8:30 a.m. – 3:30 p.m. EST
The Mayflower Hotel, Washington, D.C.

CTTI MISSION: To develop and drive adoption of practices that will increase the quality and efficiency of clinical trials

MEETING OBJECTIVES:

- Discuss disease progression modeling (DPM) and its current applications
- Explore opportunities, barriers, and best practices for advancing the use of disease progression modeling to aid in decision making
- Brainstorm relevant metrics to monitor and evaluate the recognition, value and consistent use of disease progression modeling

MARCH 6, 2023

7:30 a.m. **Breakfast** *(Provided)*

8:30 a.m. **Welcoming Remarks**

8:30 a.m. Introduction to the Clinical Trials Transformation Initiative (CTTI)
Sara Calvert, CTTI

8:40 a.m. Opening Comments
Dr. Issam Zineh, Director, Office of Clinical Pharmacology, FDA

9:00 a.m. **Session I: Project Overview and Scoping Review Results**

Session Facilitator/Moderator: *Lindsay Kehoe, CTTI*
Session Objectives:

- Provide overview of CTTI's Disease Progression Modeling project
- Present findings from project's scoping review including applications/contexts of use for disease progression modeling

9:00 a.m. Project Overview and Meeting Objectives
Lindsay Kehoe, CTTI

9:10 a.m. Project's Scoping Review Results
Summer Starling, CTTI

9:40 a.m. *Q&A*

9:50 a.m. **Break** *(Refreshments provided)*

10:05 a.m. **Session II: Challenges and Solutions for Advancing DPM Uptake**

Session Facilitator/Moderator: *Raj Madabushi, FDA*
Session Objectives:

- Explore barriers for advancing the use of disease progression modeling to aid in decision making
- Discuss essential needs to advance the use of disease progression modeling

10:05 a.m. Introduction to Session II
Raj Madabushi, FDA

10:10 a.m. Multi-Stakeholder Panel: DPM Applications & Decision Making

- Applications of DPM: Attributes and Limitations – Sponsor perspective
  CJ Musante, Pfizer
- Applications of DPM: Attributes and Limitations –Regulator perspective
  Hao Zhu, FDA
- AI/ML: Value for DPM and Adoption Challenges
  Dave Miller, Unlearn.AI
- Collaboration through Consortia: Lessons Learned
  Klaus Romero, Critical Path Institute

10:55 a.m. Q&A

11:20 a.m. Break Out Groups

Essentials to Advance DPM

- *What are the key areas that need to be addressed to incorporate DPM approaches more effectively in:*
  - *medical product development (drug, device, biologics)?*
  - *regulatory decision making?*
- *For the question above, which items can be addressed in the short term? Long-term?*

Accountability:

- *Who should be involved in driving the recognition, value, and use of DPM? What actions should they take to facilitate change?*
- *What's CTTI's role in driving the recognition, value & use of DPM?*

12:30 p.m. **Lunch** *(Provided)*

1:30 p.m. Session II Debrief
Lindsay Kehoe, CTTI

- Recap the key themes discussed in Session II

1:50 p.m. **Session III: Facilitating Progress**

Session Facilitator/Moderator: *Bruce Burnett, Duke University*
Session Objectives:

- Explore recommendations and resources needed
- Brainstorm relevant metrics to monitor and evaluate the recognition, value and consistent use of disease progression modeling

1:50 p.m. Recommendations & Resources: Open Discussion

- What recommendations and resources should CTTI develop to help advance DPM acceptance and use?
- To whom should those recommendations target?

2:30 p.m. **Break** *(Refreshments Provided)*

2:40 p.m. Metrics to Measure Change
Level setting on metrics and implementation
Sara Calvert, CTTI

2:45 p.m. Metrics Brainstorming: Open Discussion
What should CTTI be measuring to understand:
(a) whether a change in recognition, value and consistent use of disease progression modeling *is happening?*
(b) whether that change is improving the quality and efficiency of trials?

3:25 p.m. **Closing Comments**

3:25 p.m. **Highlights and Next Steps**
Lindsay Kehoe, CTTI

3:30 p.m. Adjourn

For more information, contact the Disease Progression Modeling Project Manager, Lindsay Kehoe at Lindsay.kehoe@duke.edu, or visit http://www.ctti-clinicaltrials.org.