DISEASE PROGRESSION MODELING
Expert Meeting

CLINICAL TRIALS TRANSFORMATION INITIATIVE

MEETING SUMMARY | MARCH 6, 2023

BACKGROUND

Modeling and simulations are powerful tools that can be leveraged to inform clinical trial design, support regulatory decision making, and accelerate the process of bringing treatments to patients. While the field has matured, potential for widespread use and acceptance still exists. In particular, there is growing interest across the clinical trials ecosystem in advancing the recognition and use of disease progression modeling (DPM) in order to improve clinical trial quality and efficiency.

CTTI's Disease Progression Modeling project seeks to clarify how DPM can advance decision making – including trial design, regulatory development, and business decision making – throughout the medical product development lifecycle.

As part of the CTTI project, this Expert Meeting convened a diverse group of stakeholders to review the results of the project's literature scoping review, consider attributes that allow for a successful application of DPM, and discuss recommendations needed to advance DPM use.

This work aims to advance the broader application of modeling and simulations for designing clinical trials with a quality approach that maximally leverages available data- in alignment with CTTI's **Transforming Trials 2030** vision.

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

MEETING THEMES

Throughout the meeting, attendees discussed applications and impact for DPM within clinical trials, opportunities and barriers for advancing DPM use, and the role CTTI could play in contributing to and monitoring the continued advancement of DPM. Many ideas were discussed, and the following key themes emerged:

- **Disease progression modeling is actively supporting drug development and informing clinical trial decisions.** The field of DPM has matured and is being applied across the drug development pathway, informing decisions along the way.
- **The value of DPM and its potential for impact have yet to be fully realized.** DPM has the power to transform data into knowledge and knowledge into impact. There is a need to highlight the value of DPM, share knowledge, and provide clarity around regulatory expectations in order to realize widespread use and acceptance.
- **Collaboration and communication opportunities exist to realize full potential.** Regulatory agencies have programs to foster engagement and communication around model informed drug development (MIDD) such as FDA's paired meeting program and the **Fit-for-Purpose** initiative but additional communication and pre-competitive collaboration is needed between the modeling industry, regulatory agencies, and the clinical community to advance its use.

NEXT STEPS

Meeting attendees discussed essential next steps for advancing the recognition, value, and use of DPM approaches, including the need to:

- Establish best practices and provide illustrative case examples.
- Develop a common language for disease progression modeling.
- Highlight the value and impact of DPM for a variety of stakeholders.
- Facilitate communication across stakeholders.
- Create metrics to monitor the changes in DPM acceptance and use within organizations and across the clinical trial enterprise.

The CTTI project team will use the insights provided to develop a set of recommendations and resources for the appropriate target audience.

ADDITIONAL RESOURCES

- **Meeting materials**, including agenda, participant list, and presentations
- Read more about CTTI's **Disease Progression Modeling to Advance Clinical Trial Decision Making** Project

ABOUT THE CLINICAL TRIALS TRANSFORMATION INITIATIVE (CTTI)

The Clinical Trials Transformation Initiative (**CTTI**), a public-private partnership co-founded by Duke University and the FDA, seeks to develop and drive adoption of practices that will increase the quality and efficiency of clinical trials. Bringing together organizations and individuals from across the enterprise, CTTI is transforming the clinical trials landscape by developing evidence-based solutions to clinical research challenges.

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