Duke | MARGOLIS CENTER for Health Policy

Translational Science in Drug Development: Surrogate Endpoints, Biomarkers, and More

Virtual Public Workshop

May 24, 2022 | 12:00 pm – 4:00 pm ET

May 25, 2022 | 12:00 pm – 4:00 pm ET

Workshop Agenda | Day One

As the biological mechanisms of diseases and pharmacological activities of therapeutics are better understood, this information provides opportunities to improve clinical trial efficiency. One such opportunity includes identification and use of surrogate endpoints that indicate disease progression or clinical response in clinical trials. In instances where disease progression or clinical response is slow, surrogate endpoints may provide a measurable prediction of the outcomes for clinical trials in a shorter and more feasible timeframe[1]. Development and validation of such surrogate endpoints, however, often requires sustained efforts and dedication from many stakeholders.

Surrogate endpoints represent only one way that translational science can be leveraged to support clinical development of medical products. Understanding the causal pathways of a disease can support the identification of prognostic or predictive biomarkers. Animal models of disease can provide supportive evidence for candidate therapeutics when the pathophysiology of disease is well understood and the animal model recapitulates important aspects of the human disease. When developing these types of mechanistic evidence to support a clinical development program, early discussions with regulators on the type(s) of evidence and how the evidence would be used can be beneficial.

Collaboration between academic researchers, industry, clinicians, patient organizations, and regulators can drive innovation and facilitate the use of translational science during clinical development[2]. This workshop will focus on best practices and provide use cases for successfully bringing forward evidence generated through translational science for regulatory submissions. Stakeholders will discuss potential barriers to using translational science to support therapeutic development and strategies to overcome those barriers.

12:00 pm **Welcome and Opening Remarks**

12:20 pm **Session 1: Enhancing Clinical Development Programs by Leveraging Translational Science Throughout the Drug Development Lifecycle**

Objective: In this session, speakers from academia, industry, and regulatory sectors will each present their views on the incorporation of biomarkers and other translational science into clinical development programs. Speakers will discuss the benefits and challenges of using

[1] Mack, A., et al., Perspectives on biomarker and surrogate endpoint evaluation : discussion forum summary. 2011, Washington, D.C: The National Academies Press.

[2] Luke, D.A., et al., The Translational Science Benefits Model: A New Framework for Assessing the Health and Societal Benefits of Clinical and Translational Sciences. Clin Transl Sci, 2018. 11(1): p. 77-84.

biomarkers as surrogate endpoints relative to the direct measurement of a clinical endpoint to demonstrate efficacy in clinical development.

Moderated Discussion and Q&A

1:45 pm **Break**

2:00 pm **Session 2: Identification and Development of Novel Surrogate Endpoints for Use in Clinical Development Programs**

Objective: In this session, the presenters and panelists will discuss the identification and development of biomarkers as novel surrogate endpoints that could be used in clinical development programs. The discussion will highlight common challenges during development, strategies for overcoming those challenges, and opportunities to streamline the process.

Moderated Discussion and Q&A

3:15 pm **Concluding Remarks**

3:25 pm **Adjournment**

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Workshop Agenda | Day Two

12:00 pm **Welcome and Overview of Day Two**

12:10 pm **Session 3: Clinical Validation and Regulatory Acceptance of Biomarkers as Surrogate Endpoints**

Objective: In this session, presenters and panelists will discuss the process of validating a novel surrogate endpoint for accelerated approval and traditional clinical trial settings, including common challenges during validation and solutions for overcoming them.

Moderated Discussion and Q&A

1:50 pm **Break**

2:05 pm **Session 4: Beyond Surrogate Endpoints: Other Ways Translational Science Can Support Drug Development**

Objective: In this session, speakers and panelists will present use cases beyond use of surrogate endpoints. Discussions will highlight how translational research guided the design of shorter, smaller, more efficient clinical trials and helped minimize risks to meeting efficacy and safety standards.

Moderated Discussion and Q&A

3:30pm **Session 5: Opportunities and Challenges for Incorporation of Translational Science in Clinical Development Programs**

Objective: Panelists will discuss opportunities to increase the use of translational research studies to support clinical development that achieves regulatory acceptance.

Panel and Moderated Discussion

4:15 pm **Concluding Remarks**

4:25pm **Adjournment**

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