

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

Virtual Public Workshop

May 24, 2022 | 12:00 pm – 3:45 pm ET

May 25, 2022 | 12:00 pm – 4:25 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.<sup>1</sup> 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.<sup>2</sup> 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**

**Mark McClellan**, Duke-Margolis Center for Health Policy

**Peter Stein**, US Food and Drug Administration

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

*Moderator:* **Peter Stein**, US Food and Drug Administration

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

<sup>2</sup> 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.

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

*Presentations by:*

- **Jeffrey Siegel**, US Food and Drug Administration
- **Joni L. Rutter**, National Center for Advancing Translational Sciences
- **John Wagner**, Koneksa
- **Peter Marks**, US Food and Drug Administration

**1:45 pm**      **Break**

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

*Moderator:* **Kerry Jo Lee**, US Food and Drug Administration

*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.

*Presentations by:*

- **Charles Venditti**, National Institutes of Health
- **Oleg Shchelochkov**, National Institutes of Health
- **Issam Awad**, University of Chicago
- **Steve Williams**, SomaLogic
- **Terina Martinez**, Critical Path Institute

*Panelists:*

- **Patrick Archdeacon**, US Food and Drug Administration
- **Charles Venditti**, National Institutes of Health
- **Oleg Shchelochkov**, National Institutes of Health
- **Issam Awad**, University of Chicago
- **Steve Williams**, SomaLogic
- **Terina Martinez**, Critical Path Institute

**3:35 pm**      **Concluding Remarks**

**Mark McClellan**, Duke-Margolis Center for Health Policy

**3:45 pm**      **Adjournment**

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

### Workshop Agenda | Day Two

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

**Mark McClellan**, Duke-Margolis Center for Health Policy

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

*Moderator:* **Norman Stockbridge**, US Food and Drug Administration

*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.

*Presentations by:*

- **Steve Ryder**, Rallybio Inc.
- **Henrik Zetterberg**, University of Gothenburg/University College London
- **Lesley Inker**, Tufts University
- **Nicole Gormley**, US Food and Drug Administration

*Panelists:*

- **Aliza Thompson**, US Food and Drug Administration
- **Jeff Allen**, Friends of Cancer Research
- **Steve Ryder**, Rallybio Inc.
- **Henrik Zetterberg**, University of Gothenburg/University College London
- **Lesley Inker**, Tufts University
- **Nicole Gormley**, US Food and Drug Administration

**1:50 pm**      **Break**

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

*Moderator:* **David Strauss**, US Food and Drug Administration

*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.

*Presentations by:*

- **Leslie B. Gordon**, Brown University
- **Estelle Marrer-Berger**, Roche
- **Christine Garnett**, US Food and Drug Administration

*Panelists:*

- **Lynne Yao**, US Food and Drug Administration
- **Anthony Durmowicz**, Cystic Fibrosis Foundation
- **Leslie B. Gordon**, Brown University
- **Estelle Marrer-Berger**, Roche
- **Christine Garnett**, US Food and Drug Administration

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

*Moderator:* **Michael Pacanowski**, US Food and Drug Administration

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

*Panelists:*

- **Jen Farmer**, Friedreich's Ataxia Research Alliance
- **David Reese**, Amgen
- **Steve Hoffmann**, Foundation for the National Institutes of Health
- **Jeffrey Siegel**, US Food and Drug Administration

**4:15 pm**      **Concluding Remarks**

**Michael Pacanowski**, U.S. Food and Drug Administration

**4:25pm**      **Adjournment**

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