Duke | MARGOLIS CENTER for Health Policy

Advancing the Development of Therapeutics Through Rare Disease Patient Community Engagement

Virtual Public Meeting

December 14, 2023

12:00 p.m. – 5:00 p.m. ET

Meeting Objectives

This public meeting is being convened to discuss approaches and opportunities for engaging patients, patient groups, rare disease or condition experts, and experts on small population studies during the drug development process for rare diseases. The meeting will focus on how to best understand patients' experiences living with a rare disease and how to incorporate those experiences and priorities throughout the drug development process. This includes understanding patient perspectives on the burden of their condition and any existing treatment options, as well as how their current health status and risk of disease progression may impact willingness to accept risks from treatment side effects.

12:00 pm **Welcome and Overview**

Marianne Hamilton Lopez, Duke-Margolis Center for Health Policy

12:10 pm **Opening Fireside Chat**

FDA leadership will discuss the Agency's efforts to incorporate patient and subject matter expert input throughout drug development, as well as the impetus for this meeting and potential future efforts in this space.

Marianne Hamilton Lopez, Duke-Margolis Center for Health Policy
Peter Stein, U.S. Food and Drug Administration
Nicole Verdun, U.S. Food and Drug Administration

12:30 pm **FDA Overview of Drug Review Process**

FDA will provide an overview of the drug review process and how early engagement with key stakeholders including patients and other rare disease experts can provide important information that can inform FDA's regulatory advice and decision making. The presentation will highlight how considerations of patient burden, available treatment options, and evaluation of side effects are taken into account in the development and review of drugs and biologics for rare diseases.

Teresa Buracchio, U.S. Food and Drug Administration
Robyn Bent, U.S. Food and Drug Administration

1:00 pm **Engaging Patients and Other Experts in Trial Design and Related Aspects of Drug Development**

In this session, participants will discuss common challenges and key considerations in the design of rare disease clinical trials. This session will include a discussion of opportunities for key stakeholders such as patients, patient groups, and rare disease subject matter experts to incorporate therapy- and disease-specific considerations as they plan drug development programs. Participants will also reflect on how those considerations can inform FDA efforts intended to support the drug development process. Moderated discussion will incorporate audience questions submitted live in addition to prepared questions from the moderator.

Presentation (5 min):
Michelle Campbell, U.S. Food and Drug Administration

Moderated Discussion (45 min) and Audience Q&A (20 min):
Michelle Campbell, U.S. Food and Drug Administration (Moderator)
Danielle Boyce, Tufts University School of Medicine
Emma D'Agostino, Cystic Fibrosis Foundation
Joe Horrigan, AMO Pharma
Kelley Kidwell, University of Michigan School of Public Health
Thomas Miller, Bayer
Rebecca Chiu, U.S. Food and Drug Administration

2:10 pm **BREAK**

2:20 pm **Case Studies: Engaging Patients and Other Experts Throughout the Drug Development Process**

Researchers, clinicians, patient community members, and other subject matter experts will present a series of brief case studies focused on how stakeholder engagement has helped to answer research questions at different stages across drug development.

In the following discussion session, panelists will explore approaches and opportunities to improve communication and collaboration between patients, regulators, sponsors, and other stakeholders throughout drug development. Panelists will discuss how to incorporate different stakeholder priorities to support evidence generation throughout drug development while ensuring that consideration is given to challenges such as patient burden, existing treatment options/lack of treatments, and patients' willingness to accept risks of side effects.

Presentations (30 min):
Monica Morell, U.S. Food and Drug Administration
Jennifer Farmer, Friedreich's Ataxia Research Alliance
John Sleasman, Duke University School of Medicine

Moderated Discussion (40 min) and Audience Q&A (20 min):
Gerrit Hamre, Duke-Margolis Center for Health Policy (Moderator)
Jen Farmer, Friedreich's Ataxia Research Alliance
Connie Lee, Alliance to Cure Cavernous Malformation
John Sleasman, Duke University
Lili Garrard, U.S. Food and Drug Administration
Tejashri Purohit-Sheth, U.S. Food and Drug Administration

3:50 pm **BREAK**

4:00 pm **Where We're at and Where We're Going**

In a concluding session, panelists will discuss where we are at and where we are going, including effective approaches for engagement between patients, regulators, and industry that ensures what matters to patients is considered and incorporated throughout drug development. Panelists will also reflect on further opportunities to improve meaningful patient engagement and effective communication between key stakeholders throughout drug development.

Victoria Gemme, Duke-Margolis Center for Health Policy (Moderator)
Karin Hoelzer, National Organization for Rare Disorders
Collin Hovinga, Critical Path Institute
Jennifer Panagoulias, Foundation for Angelman Syndrome Therapeutics
Saira Sultan, Haystack Project
Bobby Wiseman, Patient Advocate
Kerry Jo Lee, U.S. Food and Drug Administration

5:00 pm **Closing Remarks and Adjournment**

Marianne Hamilton Lopez, Duke-Margolis Center for Health Policy