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 virtual 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**

12:10 pm **Opening Fireside Chat**

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

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, treatment options, as relevant, and evaluation of side effects are taken into account in the development and review of drugs and biologics for rare diseases.

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

FDA will lead a discussion about 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 advocacy groups, and rare disease subject matter experts to incorporate therapy- and disease-specific considerations as they plan drug development programs. Moderated discussion will incorporate audience questions submitted live in addition to prepared questions from the moderator.

Presentation (5 min)

Moderated Discussion (45 min) and Audience Q&A (20 min)

2:10 pm **BREAK**

2:20 pm **Case Studies: Engaging Patients and Other Experts Throughout Development and Review**

Industry representatives, patients, 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 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)

Moderated Discussion (40 min) and Audience Q&A (20 min)

3:50 pm **BREAK**

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

In a concluding discussion, panelists will discuss where we are at and where we are going, including effective approaches for industry-patient engagement, ensuring what matters to patients is incorporated into drug development whenever possible, and further opportunities to engage patients and other key stakeholders throughout the drug development process.

4:50 pm **Closing Remarks**

5:00 pm **Adjournment**