

Assessing Novel Efficacy Endpoints in Ophthalmologic Rare Disease Drug and Biologics Development

Hybrid Meeting | National Press Club

September 17, 2025

9:30 am-2:30 pm ET

Agenda

This meeting, convened by the Duke-Margolis Institute for Health Policy under a cooperative agreement with the U.S. Food and Drug Administration, will focus on novel efficacy endpoints used in interventional clinical trials for drugs and biological products intended for patients with severe vision loss to support regulatory decision making. The meeting will focus in particular on full-field stimulus threshold testing (FST) and ellipsoid zone data (EZ). Researchers, clinicians, and other stakeholders will present and discuss evidence and data that may support the use of these tools in regulatory decision making.

9:30 AM **Welcome and Overview**

Brian Canter, Duke-Margolis Institute for Health Policy

9:35 AM **Patient Perspective**

This presentation will describe some of the challenges that people living with severe vision loss, as well as their caregivers, may experience on a day-to-day basis and in seeking ophthalmologic treatment. It will highlight the need for outcome measures that can capture changes in vision that matter for people with severely limited vision.

Marylee Dilling, Blue Cone Monochromacy Families Foundation

9:45 AM **Overview: Traditional and Novel Efficacy Endpoints in Ophthalmologic Therapeutic Drug and Biologics Development**

This session will provide an overview of traditional efficacy endpoints currently used in clinical trials for products treating ophthalmologic conditions and the need for novel efficacy endpoints to better capture patient outcomes particularly for rare conditions.

William Boyd, U.S. Food and Drug Administration
Ekaterini Tsilou, U.S. Food and Drug Administration

10:10 AM **Role of Full-Field Stimulus Threshold in Ophthalmologic Therapeutic Drug and Biologics Development**

Presenters will provide an overview of existing evidence supporting the use of full-field stimulus threshold (FST) testing as an efficacy endpoint in clinical trials for therapeutics intended to treat rare ophthalmologic conditions, including its clinical meaningfulness to patients. Then, panelists will discuss additional considerations for the use of FST in regulatory submissions, including the strength of the relationship between FST and traditional efficacy endpoints, whether FST change directly reflects clinically meaningful change, and other factors.

Moderator: **Brian Canter**, Duke-Margolis Institute for Health Policy

Presenters:

- **Artur Cideciyan**, University of Pennsylvania
- **Allison Ayala**, Foundation Fighting Blindness

Panelists:

- **Heidi Becker**, U.S. Food and Drug Administration
- **Jean Bennett**, University of Pennsylvania
- **William Boyd**, U.S. Food and Drug Administration
- **Thiran Jayasundera**, University of California, Davis

11:30 AM **Lunch Break**

12:30 PM **Role of Ellipsoid Zone Data in Ophthalmologic Therapeutic Drug and Biologics Development**

A presentation will provide an overview of existing evidence supporting the use of ellipsoid zone (EZ) data as an efficacy endpoint in clinical trials for drugs and biologics intended to treat rare ophthalmologic conditions, including its clinical meaningfulness to patients. Then, panelists will discuss additional considerations for the use of EZ data in regulatory submissions, including the strength of the relationship between EZ parameters and traditional efficacy endpoints, limitations or challenges in measuring EZ change, and other factors.

Moderator: **Eleonora Lad**, Duke University Medical Center

Presenter: **Justis Ehlers**, Cleveland Clinic

Panelists:

- **Amitha Domalpally**, Wisconsin Reading Center
- **Rhea Lloyd**, U.S. Food and Drug Administration
- **Omer Trivizki**, Tel Aviv Medical Center
- **Ekaterini Tsilou**, U.S. Food and Drug Administration

1:50 PM **Key Takeaways and Next Steps for FST and EZ in Interventional Clinical Trials**

In this concluding session, panelists will summarize and synthesize the most important points regarding evidence supporting the use of FST and EZ in ophthalmologic drug development for rare diseases, and will highlight next steps for use of these tools to support regulatory decision making.

Moderator: **Brian Canter**, Duke-Margolis Institute for Health Policy

Panelists:

- **Todd Durham**, Foundation Fighting Blindness
- **Onyeka Illoh**, U.S. Food and Drug Administration
- **José-Alain Sahel**, University of Pittsburgh
- **Rosa Sherafat-Kazemzadeh**, U.S. Food and Drug Administration
- **Timothy Stout**, Baylor College of Medicine
- **Dorothy Thompson**, University College London

2:30 PM **Closing Remarks and Adjournment**

Brian Canter, Duke-Margolis Institute for Health Policy

Funding Acknowledgement

This project is supported by the Food and Drug Administration (FDA) of the U.S. Department of Health and Human Services (HHS) as part of a financial assistance award U19FD006602 totaling \$5,192,495 with 100 percent funded by FDA/HHS. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement, by FDA/HHS, or the U.S. Government.