

**Endpoint Considerations to Facilitate  
Drug Development for Niemann-Pick Type C (NPC)**  
***Key Themes and Future Directions***  
***from the January 2022 Public Workshop***

Public Webinar  
August 4, 2022  
3:00 – 4:00 PM ET

**Agenda**

**Background and Webinar Objectives**

Niemann-Pick Type C (NPC) is a rare genetic disease that results in progressive neurological symptoms and organ dysfunction. NPC is caused by variants in either the *NPC1* or *NPC2* gene, resulting in impaired intracellular transport of cholesterol and other lipids. Individuals with NPC have significant unmet treatment needs. Currently, there are no approved therapies in the United States for treatment of NPC. In order to advance NPC drug development, it is important that stakeholders work together and identify strategies to support ongoing and future NPC clinical trials.

In January 2022, the Robert J. Margolis, MD, Center for Health Policy at Duke University and the U.S. Food and Drug Administration (FDA) convened a group of experts to discuss clinical endpoints relevant to clinical trials and innovative measurement strategies with the overall goal of supporting the development of safe and effective treatments for those living with NPC. This webinar will provide an overview of the key themes and future directions shared during the January 2022 workshop and presented in the recently released workshop summary report.

- 3:00 pm**      **Welcome and Overview**  
Mark McClellan, Duke-Margolis Center for Health Policy
- 3:05 pm**      **Opening Remarks from FDA**  
Patrizia Cavazzoni, U.S. Food and Drug Administration
- 3:10 pm**      **Patient Community Perspective**  
Justin Hopkin, National Niemann-Pick Disease Foundation  
Sean Kassen, Ara Parseghian Medical Research Fund
- 3:20 pm**      **Researcher Perspective**  
Forbes D. Porter, National Institutes of Health
- 3:30 pm**      **NPC Endpoint Considerations – Key Themes and Future Directions**  
Jacqueline Karp, U.S. Food and Drug Administration

**3:45 pm**

**Q&A**

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

*Respondents:*

Katie Donohue, U.S. Food and Drug Administration

Justin Hopkin, National Niemann-Pick Disease Foundation

Jacqueline Karp, U.S. Food and Drug Administration

Sean Kassen, Ara Parseghian Medical Research Fund

Naomi Knoble, U.S. Food and Drug Administration

Forbes D. Porter, National Institutes of Health

**3:55 pm**

**Closing Remarks**

Mark McClellan, Duke-Margolis Center for Health Policy

**4:00 pm**

**Adjournment**