U.S. FOOD & DRUG ADMINISTRATION

On January 24-25, 2022, FDA and Duke-Margolis will host a virtual public workshop on endpoint considerations to facilitate drug development for Niemann-Pick Type C (NPC), a rare genetic disease that results in progressive neurological symptoms and organ dysfunction. There are currently no approved therapies in the United States to treat NPC. The FDA recognizes the importance of researching and approving therapies for NPC and will continue to work with stakeholders to bring safe and effective treatments to patients in need.

The workshop will be an opportunity for participants to discuss clinical endpoints relevant to NPC clinical trials and innovative strategies to support therapeutic development for patients with NPC.

In this workshop, participants will:

- review the current state, challenges, and opportunities for endpoint selections in NPC to support product development;
- consider functional assessments that could serve as clinical endpoints in NPC clinical trials; and
- discuss innovative strategies to support product development, such as digital technology and biomarkers.

The workshop is open to the public and will include representation from the patient community, healthcare providers, industry, and other federal partners.

Please visit:
https://www.fda.gov/drugs/news-events-human-drugs/endpoint-considerations-facilitate-drug-development-niemann-pick-type-c-npc-public-workshop-01242022
for more information, the draft agenda, and to register for the upcoming workshop.

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products

Professional Affairs and Stakeholder Engagement | Office of Center Director
Center for Drug Evaluation and Research | Food and Drug Administration
10903 New Hampshire Ave | Silver Spring, MD 20993

U.S. FOOD & DRUG ADMINISTRATION