



Acid Sphingomyelinase Deficiency (ASMD) Patient Experience with Olipudase Alfa Survey

The International Niemann-Pick Disease Registry (INPDR) is conducting this survey in collaboration with The International Niemann-Pick Disease Alliance (INPDA), National Niemann-Pick Disease Foundation, Inc. (NNPDF), and Niemann-Pick UK (NPUK), who are dedicated to supporting and empowering individuals and families affected by Niemann-Pick disease, through education, collaboration and research.

This survey is part of a larger study that includes interviews with Niemann-Pick disease type A/B and type B (ASMD) patients, which will explore the questions in this survey in more depth. Interviews will be conducted by Rare Disease Research Partners (RDRP). If you are interested in taking part in an interview, please register your interest within the "Participation in interviews" section and you will receive further information.

Purpose of the survey

The purpose of the survey is to increase understanding of the impacts of olipudase alfa on adult ASMD patients and their families.

Who can take part?

The survey is open to those aged 18 years and over who:

- are fluent in English (including non-native English speakers)
- are able to give informed consent
- have a confirmed diagnosis of ASMD
- have received olipudase alfa as either an experimental or approved therapy for ASMD

Your involvement

Your participation in this survey is voluntary and you are free to withdraw at any time without notice. The survey should take no longer than 45 minutes to complete.

Risks and discomforts

We understand that answering questions concerning your own health can be upsetting. Please remember that you can stop the survey at any time. If you stop the survey and come back to it later, the answers you have already provided will be saved. You will have one week to complete the survey. When you are ready to continue the survey, simply click the survey link again using the same computer or device.

Benefits

The information gathered in this survey will serve to communicate the views, experiences, and perspective of ASMD patients and families who have experience with olipudase alfa.

It will help ensure the patient perspective is considered by thought leaders, industry, and regulators. It will also help to understand what benefits in a treatment are meaningful to ASMD patients and families as we look to promote further research and development in the field.

Confidentiality

INPDR will protect the confidentiality of information gathered during this survey in accordance with applicable data protection legislation. More information on confidentiality is provided in the informed consent portion of the survey.

What will happen to the results of the survey?

The information you provide will be shared with INPDR, NNPDF, INPDA & NPUK. Before the INPDR shares this information, we will remove your name and aggregate the data to ensure no individual can be identified. More information about what will happen to the results of the survey will be conveyed in the informed consent portion of the survey.

**If you or someone you know are interested in
participating in this survey,
please click the link below to access the survey:**

https://inpdr.qualtrics.com/jfe/form/SV_8H8sXCGVjaiKYPY