genzyme
A SANOFI COMPANY

Acid Sphingomyelinase Deficiency (ASMD)/Niemann-Pick Disease Type B (NPD B) Patient-Reported Outcome (PRO) Development

Qualitative Research Phase

Patient Interview Study Information

ABOUT PRO
Patient-Reported-Outcome (PRO) instruments are measures self-reported by patients, about disease symptoms and impact, as well as impact of treatment. When evaluating disease management, quality of care and effectiveness of new treatments, PROs are very important in understanding what is important and meaningful from patients' perspectives, and how health care interventions can be used to improve patients' health and health-related quality of life. PROs are increasingly being incorporated into clinical programs, medical practice and in observational research, to evaluate and monitor the impact of medical treatments and health interventions and thus deliver care that is most important and valuable to patients.

AIM OF PRO INITIATIVE
In the light of its commitment to patient-centricity and with the aim to continuously support the patients with Acid Sphingomyelinase Deficiency (ASMD), Genzyme a Sanofi Company has launched an initiative to develop a disease-specific ASMD/ Niemann-Pick Disease Type B (NPD B) PRO instrument. Input from the patient community is a central part of PRO development. In addition to clinical expert interviews and evidence reviews, this research initiative involves also in-depth interviews with patients with ASMD and/or their parents/ caregivers. The objective of interviews is to hear patients' and caregivers' opinion and perspectives, about the experience with symptoms of ASMD and about the impacts and burden that the disease has on their lives.

VALUE OF NEW PRO MEASURE
The newly developed PRO assessment tool will be available for use widely in various contexts and settings, including clinical research and medical practices. It will provide a comprehensive and standard tool to health care providers to measure severity, progression and overall burden of disease in patients with ASMD, as well as allow assessing and improving quality and effectiveness of care and interventions in a wider sense. This new PRO measure can also be used by researchers and other parties to understand disease impact, severity and progression of disease, as well as the value of treatments and interventions.

PATIENT INTERVIEWS
Patient input is an essential component of this initiative. Interviews will involve a one-hour discussion by an experienced scientist interviewer with patients and/or parents/caregivers. Participants will be asked questions about disease symptoms, disease impact and general questions about living with ASMD. Interviews will take place via telephone or face-to-face at a location that is convenient for participants. Participation in the interviews is absolutely voluntary and patients and/or parents/caregivers may withdraw their participation at any time.

WHO CAN PARTICIPATE
This research includes adults with ASMD, children older than 7 years of age, as well as parents/ caregivers of children with ASMD/NPD B. Your participation in the interviews for PRO measure development study does not prevent you from, or provide preference for, participating in any current or future clinical studies.

HOW TO PARTICIPATE
The interviews will be conducted by researchers from a consulting company - Evidera, on behalf of Genzyme. Evidera is required by law to ensure participants' privacy and to maintain the confidentiality of all patient level data and materials. If you are interested in learning more about this opportunity or participating in these interviews, please reach out to Evidera at the following numbers:

- US/Canada toll free number: **1-800-257-3157**
- UK toll free number: **0800-088-5390**
- You can also contact the Evidera research team at the following e-mail address: **ASMDPro@evidera.com.**

You may be enrolled in the study, once your eligibility is established.

National Niemann-Pick Disease Foundation is not involved in this study and will not have access to any of interview responses.