

Study Announcement

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

Qualitative Research Study Information

Date: August 11, 2016

ABOUT PRO

Patient-Reported-Outcome (PRO) instruments are measures self-reported by patients, generally about disease symptoms and impact, as well as the impact of treatment. When evaluating disease impact, management and quality of care and effectiveness of new treatments, PROs are essential 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 meaningful and valuable to patients.

AIM OF PRO INITIATIVE

There are currently no ASMD disease-specific PRO instruments, and no PRO questionnaires have been validated in patients with ASMD. In addition to limited PRO-based evidence in ASMD studies, there are known limitations of generic instruments in providing useful quantitative data. To address this need, Sanofi Genzyme has initiated a project for developing and/ or validating PRO measures for patients with ASMD. This work aims to understand the disease impact and/ or value of treatment from the patients' perspective, and ensure that outcomes that are meaningful to patients are evaluated in future studies. The specific aim of this research is to test with patients the new PRO measures developed during the first phase of the qualitative research, to ensure they are appropriate and suitable for completion by the ASMD population.

VALUE OF THE PRO MEASURES

. The newly developed PRO aims to 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 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 up to a two-hour discussion (either one 2-hour session, or two 1-hour sessions) between an experienced

scientist interviewer and a patient. Upon screening and enrollment into the study, participants will be given questionnaires to complete and return to the site, followed by a subsequent interview to share their views on clarity of the items/questions and instructions, interpretation of items, ease of completion of the questionnaires and comprehensiveness of the topics. Interviews will take place face-to-face at a location that is convenient for participants or via telephone. Participation in the interviews is absolutely voluntary and patients may withdraw their participation at any time.

WHO CAN PARTICIPATE

This research includes adults with ASMD and also adolescents 16 years of age and older. Please note that your participation in this interview study does not prevent you from, or provide preference for, participating in any current or future clinical studies. Patients who participated in the first phase of the qualitative research are also eligible to participate in this phase of research.

HOW TO PARTICIPATE

The interviews will be conducted by researchers from a research consulting company - Evidera, on behalf of Sanofi 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 and participating in the interviews, please reach out to Evidera at the following numbers: US/Canada toll free number: 1-800-257-3157, and UK toll free number: 0800-088-5390. You can also contact the Evidera research team at the following e-mail address: ASMDPro@evidera.com. Once your eligibility is established, you may be enrolled in the Qualitative Research study.

The National Niemann-Pick Disease Foundation, Inc. (NNPDF) is not involved in this study and will not have access to any of interview responses and individual data.