



INFORMATION SHEET

You are being invited to take part in a survey being conducted by Rare Disease Research Partners (RDRP) on behalf of Niemann-Pick UK (NPUK). The survey is entitled “**Niemann-Pick disease type C (NPC) patient and caregiver experience**”. Before you decide whether to take part please take time to read this information carefully and discuss it with others if you wish.

Purpose of the survey

We know that Niemann-Pick Type C (NPC) has an effect on quality of life for patients and carers. The purpose of the survey is to increase understanding of the impacts of NPC on patients and their families and to explore the effects of any treatments received.

This is a critical time for our community, with several potential therapies at different stages of development. To help shape their evaluation of a potential therapy and whether to approve its use, decision makers will assess the patient experience alongside other elements, such as effectiveness and safety of new medicines.

As their decisions profoundly affect the treatment and care made available to patients, it is essential that the information they receive accurately reflects the views and experiences of patients and families affected by NPC, along with your expectations of new treatments.

By participating in this survey, you will have the opportunity to input into the decision making process and to help improve the understanding of how NPC affects the lives of patients and their families by sharing your unique insights.

This survey forms part of a larger study which includes interviews with NPC patients and their caregivers, which will explore the questions in this survey in more depth. If you are interested in taking part in an interview, please register your interest within the "Participation in interviews" section of the survey and RDRP will send you further information.

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) and
- have a confirmed diagnosis of NPC or,
- are the parent or carer of a person with a confirmed diagnosis of NPC or,
- are a bereaved parent or carer of a person with a confirmed diagnosis of NPC

Do I/we have to take part?

No, it is up to you to decide whether or not to take part. If you decide to take part, you will be asked to keep this information sheet and to provide your consent to take part. If you decide to take part and then change your mind, you are free to withdraw (stop taking part in the study) at any time during the survey or the interview, without giving a reason. Whether you decide to take part or not will not affect the support you receive from NPUK.

What are the benefits of taking part?

The information gathered in this survey and through the optional interviews will help NPUK to understand and communicate the patient's perspective of NPC, its impact on that person and their family plus their views and experience of current and future treatments. It will help them to advocate for access to new and existing treatments here in the UK and around the world, and provide evidence to support further research.

How much time will the survey / interview take?

The online survey should take no longer than one hour to complete. If you would prefer to complete the survey on paper or over the telephone, please contact RDRP at info@rd-rp.com. If you also decide to take part in an interview, this may last up to 90 minutes, not including any breaks..

Will I be reimbursed for my time?

Participants may be eligible to receive compensation in the form of a gift voucher, once survey completion of the survey has been verified.

What are the disadvantages or risks of taking part?

We understand that answering questions concerning your own or your child's health can be upsetting. Please remember that you can stop the survey at any time. You can also 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.

If you take part in an interview, this can be stopped at any time you wish and you can either take a break or decide you do not want to take part anymore.

You can also request the support of a liaison person from Niemann-Pick UK or your local patient advocacy group, who can be available to help before and after the survey/interview.

What do I/we need to do if I/we decide to take part?

If you would like to take part in the online survey you can do so via the following link:

https://mpscom.eu.qualtrics.com/jfe/form/SV_cU31fbkNGz9P7Tg

To take part in an interview, please enter your e-mail address within the "Participation in interviews" section of the online survey and RDRP will send you further information.

If you would prefer to complete the survey on paper or over the telephone, please contact RDRP at info@rd-rp.com

Will the information I provide be kept confidential?

RDRP conduct their research in accordance with the British Healthcare Business Intelligence Association's Legal & Ethical Guidelines. They will protect the confidentiality of information gathered during the survey and interviews in accordance with applicable data protection legislation. You can view their data protection policy here:

www.rd-rp.com

Your personal information will be used for the purposes of conducting the survey/interview and will be kept for no longer than is necessary in accordance with their data retention policy. You can view our data retention policy here:

www.rd-rp.com

The information you provide will be shared with NPUK. Before your information is shared with NPUK, RDRP will remove your name and aggregate the data. This means that your answers to this survey will be combined with the answers from other participants. We would like to share anonymised direct quotes from your answers. If you would prefer us not to share your direct quotes you can indicate this in the 'Consent' section of the survey.

What will happen to the results of the study?

NPUK may use the data collected in this survey in the following ways:

- To provide evidence for the National Institute of Health Care Excellence's (NICE) evaluation of future treatments
- For evidence, research or other purposes that support the global NPC patient community
- To advocate for access to new and existing treatments in the UK and around the world, and provide evidence to support further research
- The anonymised results of this research may be presented at meetings or in reports or publications. Your/your child's identity will not be disclosed in those presentations or publications.

Who has reviewed the study?

RDRP are a corporate member of the British Healthcare Business Intelligence Association and conduct research in accordance with their Legal and Ethical Guidelines. In addition to their expert team, this study has been reviewed by the following health, research and patient advocacy professionals:

- Dr William Evans, GP and Chair of NPUK
- Jacqueline Imrie, NPUK Trustee, retired Clinical Nurse Specialist and Clinical Research Manager, International Niemann-Pick Disease Registry
- Conan Donnelly, PhD, Chief Executive Officer, International Niemann-Pick Disease Registry
- Laura Bell, Niemann-Pick Clinical Nurse Specialist, NPUK
- Helen Carter, Trustee and Patient Representative, NPUK
- Toni Mathieson, Chief Executive Officer, Niemann-Pick UK

Can I get more information?

If you have any questions or require more information before you decide whether or not to take part, please contact:

RDRP

Email: info@rd-rp.com

Tel: 0345 260 1087

Rare Disease Research Partners

MPS House, Repton Place, White Lion Road, Amersham, HP7 9LP

NPUK

Email: info@npuk.org

Tel: 0191 415 0693

Niemann-Pick UK

Suite 2, Vermont House, Concord, Washington, NE37 2 SQ

Thank you for taking time to read this information sheet.