



PREFER project: **Quantifying patient preferences in neuromuscular disorders (online survey).**

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We would like to invite you to take part in a research study. Before you decide whether to take part, we ask that you read this information sheet carefully. If you have any questions, please feel free to contact us via the email address shown above.

What is the purpose of the study?

The purpose of this study is to collect information on patients' views and preferences for potential treatments in neuromuscular disorders. In order to collect this information we will ask participants to complete an online survey that will be sent out in stages.

Who can participate?

We are inviting patients and caregivers from the **myotonic dystrophy type 1** and the **mitochondrial disorders** community.

If you are interested in participating **as a patient**, you must have a diagnosis of either myotonic dystrophy type 1 or a mitochondrial disorder and be 18 years old or older.

If you are interested in participating **as a caregiver**, you will need to meet the following criteria:

-Either the spouse, partner, parent, legal guardian, close relative or other adult close to the patient who, within the last 12 months, has been in contact with the patient in a caregiver relationship at least 4 times/week for at least one hour or more per day.

- As a caregiver you may have been diagnosed with the disease yourself, however you are choosing to respond for someone else at this time.
- You as caregiver should be 18 years or older but the patient being cared for can be of any age.

Who is running the study?

Researchers at Newcastle University are running this study, which is part of a larger project- called PREFER, involving a number of other partners (listed at the end of this document).

Is this study scientifically and ethically justified?

The Newcastle University research ethics committee reviewed the proposal of this research project to ensure that it is scientifically and ethically justified and that the rights and wellbeing of participants are respected.

Do I have to participate?

Participation is voluntary. You can say no to taking part without giving a reason and without it affecting your rights.

What will be involved with participation?

As part of this study you will be asked to provide an email address, which we will store and use only for the purposes of this study. We will use your email address to contact you directly with the surveys at each applicable stage.

An initial brief questionnaire will help us check that you are suitable (or eligible) for the study (refer to 'Who can participate?' section for details). After confirming eligibility, you will receive a survey split into two stages. Each section will be emailed to you directly, with a period of approximately 2 weeks in between emails.

We expect each survey stage to take about 30 minutes to complete, however you can close the survey at any time and come back to where you left off. You can also leave the study and stop participating at any time.

Where can I answer this survey?

There is no restriction on where you can complete the survey as long as you are using your personal email account to log in into it. However, we recommend using a bigger screen such as a computer / tablet to complete the survey rather than a smartphone.

What benefit will I get from participating?

There is no expected direct benefit for taking part in this study. However, we hope this study will allow researchers to better understand patients' points of view on potential treatments for their condition. We also hope the study will help inform medical professionals and companies when they make decisions about developing or offering medical treatments.

As an appreciation for your time, PREFER will offer you the option of either an Amazon voucher or a donation to a Patient Organisation of your choice (as listed at the end of this document). These will be emailed to you after survey completion.

Are there any risks or discomforts that I should expect from this participation?

As this is an online survey that can be completed from home or any location you feel comfortable at, there are no expected risks associated with your participation. However, if you feel uncomfortable when completing the survey or as a result of it, you can contact the study coordinator.

How will my personal data be kept confidential?

Every participant will receive a unique code that will identify them on the study dataset (i.e. coded data). Information that could lead to your identification (e.g. *email address*) will only be safely stored at Newcastle University in the United Kingdom. This process is called coding and from the coded (pseudonymised) data you cannot be identified. This means that your personal identifying information will never be included in reports or publications **nor** visualized by third parties. The data collected during the study will be stored in a secured database at Newcastle University and Uppsala University. The study will adhere to the local data protection laws, in this case PREFER adheres to the European General Data Protection Regulation (GDPR) 2016/679. This means that your identity and that of other participants will be kept strictly confidential, it also means that you have certain rights over your personal data.

How will my coded data be used?

Your coded data will be used to analyse the study results. After data-collection has been completed and the data is ready for analysis, the coded data will be analysed not only by researchers at Newcastle University but also by other case-study partners (as listed at the end of the document) to support the analyses.

Partners of the PREFER project (including other universities, industry partners, patient organizations and regulatory authorities as listed at the end of the document) will use this data on their intent to publish the results in scientific journals and to disseminate them via presentations at congresses or meetings. However, no identifiable data will be published.

How long will my personal data be stored?

Records containing your identifiable personal data (i.e. email account) and your coded data will be retained at Newcastle University (in UK) and at Uppsala University (in Sweden) for up to 15 years after the end of this study.

What rights do I have concerning my personal data?

If you would like to review, correct, update, restrict, object to the processing or delete personal data, or if you would like to receive an electronic copy of the personal data you have provided, you should contact one of the people mentioned at the top of this form. Your request for data deletion will be addressed within 30 days after your request has been confirmed. Such request may not be fulfilled in case that deletion renders or seriously impairs the study objectives, or in the case that regulations and laws that apply to this research require this data to be retained. Please note that you may not be able to review some of the data until after the end of the study. You can request to forward any questions, concerns or complaints about your data to the Information Security Officer at Newcastle University: rec-man@ncl.ac.uk.

What will happen at the end of the study?

When this study finishes and the results summarized, these will be available through your local Patient Organization.

Please contact the study coordinator mentioned at the top of this form for any questions regarding this study or to confirm your participation.

Thank you for your interest and participation!

List of Neuromuscular Disorders case-study partners:

Academic Partners: Newcastle University, Uppsala University, Erasmus University Rotterdam, University Medical Centre Utrecht, University of Leuven, Newcastle University.

Industry Partners: Merck KGaA, MindBytes, Janssen, Eli Lilly and Company, Sanofi, Bayer and Novartis.

Patient Organizations: Muscular Dystrophy UK (MDUK), CureDM, Lily Foundation in the UK, United Mitochondrial Disease Foundation (UMDF), MitoCanada; Muscular Dystrophy Canada (MDC), Muscular Dystrophy Association (MDA), Myotonic Dystrophy Support Group (MDSG), Mito Foundation, and Muscular Dystrophy New Zealand (MDNZ).

Full list of PREFER partners and stakeholders:

Academic Partners: Uppsala University (co-ordinator), Erasmus University Rotterdam, European Institute of Oncology, National Cancer Research Center G Paolo II, University Medical Centre Utrecht, University of Birmingham, University of Erlangen, University of Leuven, Newcastle University.

Industry Partners: Novartis Pharma (project leader), AbbVie, Actelion Pharmaceuticals, Amgen, Astellas Pharma Europe, AstraZeneca, Bayer, CSL Behring, Eli Lilly and Company, Janssen, Merck KGaA, MSD, Roche, Sanofi, Takeda Development Centre Europe.

Patient Organizations: Muscular Dystrophy UK (MDUK), European Patients Forum (EPF), International Alliance of Patients' Organizations (IAPO), European Cancer Patient Coalition (ECPC).

HTA-bodies: Belgian Health Care Knowledge Centre (KCE), Canadian Health Technology Assessment Agency (CADTH), Belgian National Institute for Health and Disability Insurance (NIHDI), Gemeinsamer Bundesausschuss (G-BA; the German reimbursement agency), the Austrian Ludwig Boltzmann Institute, the European network for HTA (EUnetHTA).

Regulators: European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA) CBER division.

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