# PREFER project:

**Patient and caregiver unmet health priorities and risk tolerance for neuromuscular treatments (qualitative phase – focus groups discussion based).**

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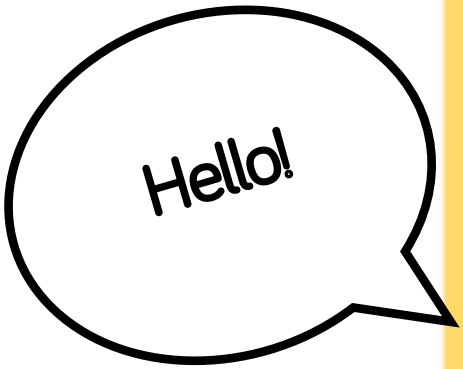
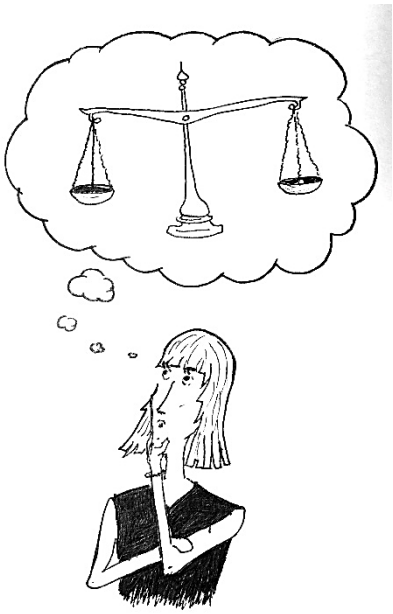
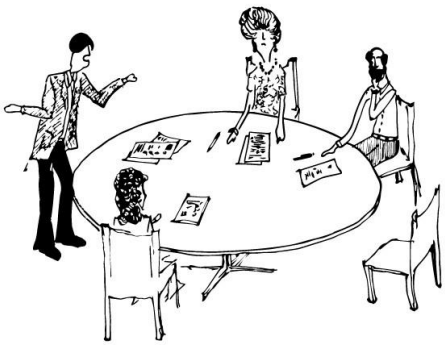



You are being invited to take part in a study exploring patients health priorities and their views on the risks and benefits of potential neuromuscular treatments.

We are interested on your views and preferences for potential treatments for the disease that affects you or the patient that you care for (for the purposes of this study could be either myotonic dystrophy or a mitochondrial disorder).

Before you confirm your participation in this study, we ask you to read this information sheet carefully. This sheet will inform you about the project itself and what would be expected during your participation. For any questions regarding this study, please contact the research coordinator.






The purpose of this study is to explore your input regarding health issues that are a priority for you (or the patient that you care of) and the expectations you (or the patient that you care of) have for possible treatments.

These group discussions will allow researchers to develop a questionnaire will then be used in a large survey of patients and caregivers (with either myotonic dystrophy or mitochondrial disorders).

This study is conducted by researchers at Newcastle University in collaboration with international partners (i.e. University Medical Center Utrecht, MSD, Actelion, Erasmus University, University of Leuven, Janssen R&D, and, Muscular Dystrophy UK (MDUK), among others). This study is part of a project called "The Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle" (PREFER)". The PREFER project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 115966. This Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.


You have been invited to consider this study as you have been identified as either a patient or a caregiver of a patient with myotonic dystrophy or a type of mitochondrial disorder.

We have selected these two diseases as both represent neuromuscular disorders that affect different body parts, both are consider rare diseases that can affect more than one family member, and both have no approved medicines to relieve symptoms.

## Do I have to participate?

No, your participation is completely voluntary. You can refuse to participate at any time. If you decide not to participate, this will not affect your (or your patient's) current or future health care.

If you decide to participate, you:

1) are still free to leave the study at any point, without giving any reason.
2) do not have to answer any questions that you do not feel comfortable answering.
3) do not have to pay anything to take part.

## What will be asked from us?

For this study you will participate in **one or more focus group discussions** with a member of the research team.

These discussions will all involve discussions around a hypothetical medication, however no treatment will be offered as part of the study. Each session is expected to last between **1 and 2 hours**, but it can be ended before if needed or if you ask for it. There will be break in the middle. The session will be conducted in an informal place that will allow the group to feel comfortable. If you are still interested in participating, we will contact you to schedule you for one of these sessions.

During the discussion, information will be gathered on your (and your patient's) health status, age, gender, socioeconomic status and about your personal treatment preferences.

To facilitate the researchers' note-taking, the interview will be audio recorded and later on transcribed (written down) into an electronic version.

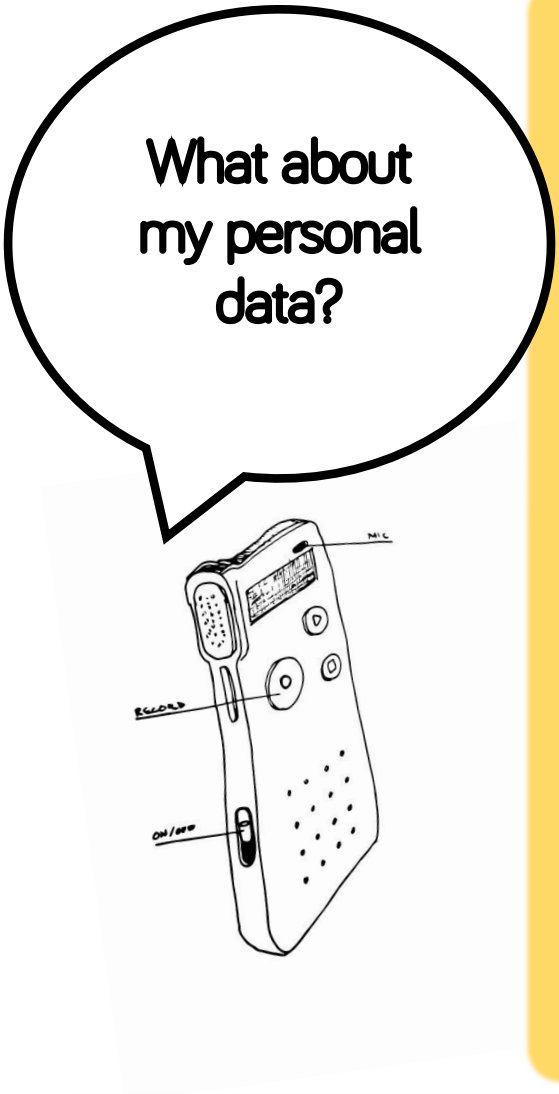
## Would this research benefit me?

It is unlikely that taking part in this study will help you directly. However, many people enjoy talking to researchers about their experiences. The insight provided by you will certainly help us (researchers) to continue informing the PREFER project and to understand better health issues that are priority for you and your expectations for possible treatments.

To thank you for your time and participation, we will give you £15.00 worth of shopping vouchers at the end of your participation.

## Are there any risks or disadvantages?

As there is no intervention involved with this study, we do not expect any risks with participating. However, if you encounter any situation along the study that discomforts you, please do not hesitate to raise the issue with one of the researchers attending the session.




How will my personal data be kept confidential? The data collected during this study will be stored in a secured database at Newcastle University as well as at the PREFER central storage at Leuven University, Belgium and for long-term storage at the Uppsala University, Sweden.

The study will adhere to the national and local data protection laws. Your identity and that of other participants will be kept strictly confidential. In the secured database where the information of all participants are kept and analyzed, information that could lead to your identification (e.g. full name, date of birth or contact details) are removed and replaced by a number at time of the data transcription. This process is called coding and from the coded ('pseudonymized') data you cannot be identified; thus, no reporting of your personal identifying information in reports or publications can happen.

How long will my personal data be stored?
Records containing your personal data and your coded data will be retained at the study site (Newcastle University) as well at Leuven University for the short term and then transferred for long-term storage at the Uppsala University with a total maximum storage time of 15 years after study end.

How will my coded (pseudonymized) data be used?
Your coded data will be used to learn more about how patients perceive their condition, treatment options and how they make choices between alternatives. In addition, your coded data may be used to answer future scientific research questions by PREFER partners, support submission dossiers to regulators, and in the design of future studies.

## 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 persons mentioned at the top of this form. Your request for data deletion will be addressed within 30 days after your request have 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, and a request to delete your personal data cannot be fulfilled in case regulations and laws require your personal data to be retained. You can request the contact persons mentioned at the top of this form to forward any questions, concerns or complaints you may have to the data protection officer of their institution. You also have the right to lodge a complaint to the data protection authority at Newcastle University with an email to the Information Security Officer: rec-man@ncl.ac.uk

## How will my coded data be shared and transferred?

The researchers may share your coded data with PREFER partners, regulatory authorities as well as with partners with whom it is working to jointly conduct scientific research in other countries. The data protection laws in these countries may be less protective than data protection laws in the European Economic Area (EEA). With regards to transfers from the EEA to other countries, for example the U.S., standards will be followed that have been established by the European General Data Protection Regulation (GDPR), national and local laws.


The results will be analyzed by researchers and health care professionals. These results will support continued research by the PREFER project and will allow the dissemination of results. via publications in scientific journals and presentations at congresses or meetings.
In addition, at the end of the project, researchers will inform relevant stakeholders (i.e. PREFER partners and patient organisations) about the results. No identifiable data will be published without your specific consent.


Ethics committees verify if the rights of participants are respected by researchers during a study, if the balance between risks and benefits is beneficial for the participants and if the study is scientifically and ethically justified.
This study complies with the International Harmonised Guidelines for Good Clinical Practice. This study has been revised and approved by the Policy & Information Team, Newcastle University Research Office.

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

**Thank you for your interest and participation!**