

The ATP study

Global Research Study to Understand the Impact of TK2d on Patients and Caregivers

PARTICIPANT INFORMATION SHEET

INTRODUCTION

You have been invited to take a survey about living with TK2d. Your participation is voluntary. If you agree to complete the survey, you will be asked to sign a consent form that states how your responses and personal information will be used. Please take as much time as you need to read the information below to help you to decide whether you would like to take part.

WHAT IS THE PURPOSE OF THIS SURVEY?

To better understand what it is really like to live with TK2d or care for someone with TK2d; including the physical signs and symptoms, and the impacts of living with, or providing care for someone with, TK2d.

The survey will be conducted in North America, Europe and South America, and will involve up to 100 participants. The results of the survey will be shared at medical conferences and in scientific journals.

WHO IS CONDUCTING THIS SURVEY?

This survey is being conducted by Rare Disease Research Partners (RDRP) on behalf of a pharmaceutical company, in partnership with several organizations who work to support patients and families living with TK2d, including International Mito Patients (IMP), the United Mitochondrial Disease Foundation (UMDF), MitoAction, the Lily Foundation and the Association contre les Maladies Mitochondriales (AMMi). The identity of the pharmaceutical company will be revealed at the end of the survey. The survey will be conducted under all appropriate regulations, specifically those of the ABPI (Association of the British Pharmaceutical Industry).

WHO CAN TAKE PART?

The survey is open to:

- adults with TK2d
- caregivers (parents or other caregivers) of a person with TK2d
- caregivers (parents or other caregivers) of a person with TK2d who died after 2008

Those completing the survey must:

- be aged 18 years of age or older
- be able to give informed consent
- have a genetically confirmed diagnosis of TK2d, or be the caregiver or bereaved caregiver of a child or adult with a genetically confirmed diagnosis (e.g. by genetic testing)
- be able to complete the survey online, using any device
- be fluent speakers of the native language of the survey and able to read, write and fully understand the language

WHO CANNOT TAKE PART?

If the person with TK2d has **received treatment with nucleoside therapy on a clinical trial** (the MT1621 study, with signed informed consent), please do not complete the survey.

NOTE: *If the person with TK2d has received nucleoside therapy outside of a clinical trial (i.e. via compassionate use, or another means, such as self-pay or other access with non-pharmaceutical grade nucleoside therapy), you can complete the survey.*

YOUR INVOLVEMENT

After providing consent, you will be asked three questions to confirm whether you are eligible. If you agree to take part and are eligible, you will then be able to complete the survey. The survey includes two parts, you may choose the sections that you would like to complete, and the survey will guide you to the appropriate sections:

The patient section

- These questions are about the experience of the person with TK2d
- It can be completed by the person with TK2d or a caregiver if the person with TK2d is too young or unable to complete it themselves

The caregiver section:

- These questions are about the experience of being a caregiver

COMPLETING THE SURVEY

The survey should take around 60 minutes to complete. You do not have to complete the survey all at once. You may close the survey and complete it at another time. To do this, you will need to 'accept cookies' if prompted by your internet browser. This will save your progress in the survey. You will also need to complete the survey using the same device that you started it on.

Once you complete the survey, you will be given the option to review and download a copy of your signed Consent Form and your responses to the survey questions.

If you wish to, you will also receive a €60 (or local currency equivalent) gift voucher for your time spent completing the survey in full and a summary of the findings once analysis is complete.

POTENTIAL BENEFITS OF TAKING PART

By sharing what we learn about the experiences of people living with TK2d and their caregivers, we can raise awareness about the condition with the wider patient, medical and scientific community.

POTENTIAL RISKS AND DISCOMFORTS

While there are no known risks or discomforts associated with taking part in this survey, you may feel uncomfortable or upset answering some of the questions, since we ask you about your experience of TK2d or your experience of caring for someone with TK2d. Please remember that you may stop the survey at any time.

Support is available for those living with TK2d should you need it:

<https://www.mitopatients.org/about/members>

WHAT IF YOU CHOOSE NOT TO PARTICIPATE IN THE SURVEY?

Your decision to take part in this survey is completely voluntary. There will not be any consequence to you if you decide not to take part.

HOW TO WITHDRAW FROM THE SURVEY

If you decide to take part, you are still free to withdraw (stop the survey and ask for your response to be deleted) at any time without giving a reason. To withdraw from the survey, please contact RDRP, our contact details are given below. Please note that if you would like to withdraw after completing the survey, we will only be able to delete your response if it has not yet been

anonymized, excluding your answers after anonymization will not be possible.

To withdraw please contact us on: info@rd-rp.com

Rare Disease Research Partners MPS House, Repton Place, White Lion Road, Amersham, Buckinghamshire, HP7 9LP, UK t: [+44 \(0\) 345 260 1087](tel:+441494345260) w: rd-rp.com

CONFIDENTIALITY

The information you provide in the survey will be kept confidential by RDRP, in accordance with applicable data protection legislation and the British Healthcare Business Intelligence Association's Legal & Ethical Guidelines.

You can view our Data Protection and Privacy Policy here:

https://www.rd-rp.com/s/1-BC_202308_Data-Protection-and-Privacy-Policy_Approved_v30.pdf

We may occasionally transfer your personal information to countries which do not offer the same level of data protection as the UK. We will require that any such organizations agree to comply with our Data Protection and Privacy Policy before any information is transferred to them.

You can complete the survey anonymously but will have the option to provide your email address for the purpose of payment, to receive an overview of the survey findings, and to receive information about future research. Your email address will not be shared with the sponsoring company.

Responses will be anonymized by assigning a unique identification number to each response and your email address (if provided) will be removed from the dataset so that any information you provide will not be linked directly to you.

What happens to my information if I do not pass the screening questions?

If the screening questions indicate that you are not eligible to take part in this survey you may be given the option to provide your email address to be contacted about future research, this is optional, and you will only be contacted if you give your permission to do this. Your email address will not be shared with the sponsoring company.

How long will the information I provide be kept?

Your personal information will be used for the purposes of conducting the research and will be kept for no longer than is necessary in accordance with our data retention policy.

After the survey closes, the anonymized responses will be archived for 5 years by RDRP, after which they will be destroyed. The archived data will be stored on RDRP's secure server.

If you have provided your email address, this will only be used for the specific purposes you have given us consent for. Email addresses will be stored separately from your survey responses, on RDRP's secure server and destroyed 5 years after the survey closes.

You can view our Data Retention Policy here:

https://www.rd-rp.com/s/Data_Retention_Disposal_Policy_2022.pdf

HOW WILL MY INFORMATION BE SHARED AND USED?

Only the RDRP research team will have access to your survey responses before they are anonymized. Initials, email addresses, age, country and any reference to a person's name or location you have given in your answers will be removed before the responses are shared with the

sponsoring company. We would like to share anonymized direct quotes from your answers. If you would prefer us not to share your direct quotes you can indicate this in the consent form.

The sponsoring company may use the anonymized data from this survey for research and development purposes in relation to their products including, without limitation, as evidence or support in connection with reimbursement and similar discussions with customers and payers.

Your email address will not be shared with the sponsoring company. RDRP will only contact you if you consent to be contacted to receive an overview of the survey findings, gift card or information about future research.

Publication

Your anonymized survey responses will be combined with the answers from other participants. These combined survey results may be presented at meetings or in reports or publications. Your identity will not be disclosed in those presentations or publications.

ADVERSE EVENT REPORTING

During the survey, if you give details of side effects or product complaints relating to one of the sponsoring company's products, we must report this information to them. This is to help them learn more about the safety of their medicines. The details will be reported anonymously unless you agree that we can share your e-mail address with the sponsoring company for the purpose of follow-up by their drug safety team. Giving your consent to share your e-mail address is optional and will have no impact on the confidentiality and anonymity of the survey itself.

COMPLAINTS AND QUESTIONS

Before proceeding to the survey, please ensure that you have taken as much time as you need to read the information provided and decide if you would like to take part.

If you have any questions, concerns or complaints about the survey please contact us on:

info@rd-rp.com

Rare Disease Research Partners

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Click on the link below to access the survey as...

[a person with TK2d](#)

[a caregiver of a person with TK2d who is still alive](#)

[a caregiver of a person with Tk2d who has died](#)