Information leaflet for participating in the MCT8 deficiency (AHDS) patient registry

For the purpose of this Consent form "the patient" will refer to the person diagnosed with MCT8 deficiency. "You" will refer to the person entering the information: this may be the affected individual or a family member or guardian of the affected individual (the person legally responsible for the care and maintenance of the affected individual) or the doctor caring for the patient. "The registry administrator" refers to authorized people in the Rotterdam Thyroid Center, associated to the Erasmus Medical Center Rotterdam (The Netherlands), who are responsible for the registry.

A patient registry is a place where medical information, family history and other related information from patients is collected and stored for medical research. The purpose of the MCT8 deficiency registry is to collect and store medical information and other information from as many as possible individuals affected by MCT8 deficiency.

The information in the MCT8 deficiency registry will be used for medical research to better understand how MCT8 deficiency affects people. This is important to optimize the care for each individual MCT8 deficiency patient (e.g. what symptoms commonly occur and have to be monitored and to develop new treatment options). The registry also addresses other critical needs. For example, scientists who want to start studies, such as to test new treatments will need access to the MCT8 deficiency registry to locate people who may be eligible to participate in such studies. The MCT8 deficiency registry is currently not sponsored, but there are no costs involved in participating.

If you join the registry, you will be asked to provide medical information on your child's disease and diagnostic process by yourself and/or by the doctor caring for the patient. The goal of the registry is to share detailed medical and other information with scientists and other researchers, while still protecting your privacy. This is done by hiding the name, address and other "identifying" information from the researchers. We call this information "de-identified" because it has been removed of all personal identifiers. Your personal information such as, your name, address, or other information that identifies you or your family will be labeled with a code number and stored in a secure place and protected with a password. Only the registry administrator will know the code and be able to identify you if needed.
Your identifiable information will not be shared with anyone beside the registry administrator (unless you give your permission to share it). Approved scientists, researchers, and clinicians, will be allowed to see only the de-identified information and may search the de-identified data for patients for their studies. If a patient looks like a good match for a study and a researcher wants, to contact you, he can do it only through the registry administrator. The MCT8 deficiency registry administrator or registry's agent (such as genetic counselor or your child`s medical doctor) will then contact you but the researcher will not contact you directly.

Your de-identified information (information that has been removed of all identifiers) may be shared with other databases such as the Global Rare Disease Patient Registry and Data Repository (GRDR) in the future. In that case, data may be protected under different laws compared to EU or Dutch laws.

The Rotterdam Thyroid Center, associated to the Erasmus Medical Center Rotterdam (The Netherlands) is the guardian of the information contained within the registry. Only approved members of the Rotterdam Thyroid Center will have access to the de-identification code. The registry guardian is responsible for proper information storage according to all international laws, select researchers and medical doctors that may access to the de-identified information and keep the registry up-to-date. The data will not be shared with pharmaceutical companies. The data will be stored indefinitively.

The MCT8 deficiency registry will also be linked to a biobank in the (near) future. This biobank is a place that will store tissue, blood or other samples that have been collected from MCT8 deficiency patients. These (anonymized) samples can then be used for research purposes. You may be asked to donate your samples to the biobank. If you decide to donate your samples, you will need to provide separate consent for the biobank.

Providing your child`s information to the registry, or contributing blood or other samples to the biobank is voluntary. Participation may not benefit your child personally, medically or financially. However, your participation may help other (yet to be diagnosed) individuals with MCT8 deficiency or related diseases by increasing the understanding of MCT8 deficiency. The collected data may help to speed up research by collecting information scientists can use. Researchers may learn how treatments work and what medical problems need to be addressed. Medical professionals may be able to improve how they treat the disease by learning from the experiences of others. In addition, participants may receive information about opportunities to participate in research (such as clinical trials), medical advances and other news from the registry. Frequent updates of important findings will be provided through the MCT8 deficiency newsletter and www.mct8.info.

Should you change your mind and wish to withdraw your data from the registry, you will be free to do so without having to provide any explanation. Simply contact the registry and all of your data will be removed from the database. Data assigned to a specific study prior to your request for removal cannot be retrieved from researchers that have already accessed it.

There is minimal risk in taking part in the registry. The registry includes questions that can be sensitive and you may feel uncomfortable answering. You do not have to share any information you do not want to. Another unlikely risk is potential breaches in the computer system. In the event there is a breach in the registry's computer system all participants will be notified.
Registry information will be collected on all patients who are diagnosed with MCT8 deficiency. The legal guardian or parent of the patient must sign the consent for the patient to join. When the patient becomes 16 (and if they are able), consent will be obtained directly from them for continued participation.
You will be asked to update your registry information at least once per year. The registry will send you a questionnaire regularly. The registry may also ask you to fax or upload your genetic test results, and any other relevant reports or testing results. The registry will also ask your doctor to provide information from the patient's records including but not limited to the disease history, genetic test results, biochemical tests and imaging data. Once available, your registry account can be updated whenever there is a change in the patient's health, change in medication, or new symptom. If the registry cannot contact you, your account may become inactive.

Other common questions:

Who do I contact with questions?

If you have any questions about the registration process or about participation in the registry, please contact the registry at (***mct8.deficiency@erasmusmc.nl***). To report concerns that result from your participation in the registry, you may contact the registry at (***mct8.deficiency@erasmusmc.nl***).

I want to be involved in a clinical trial. If I register, is this guaranteed?

Although one of the main goals of the registry is to make it easier for affected individuals to participate in research, there is no guarantee that those participants will be eligible for a trial.

Please note that even if the coordinators of a clinical trial believe that you might be eligible for the trial, based on the data stored in The Registry, it is still possible that later on it will turn out that you do not meet the trial requirement criteria after all. Please also be aware that if we inform you about the existence of a trial, this does not imply that we endorse it. In order to participate in any trial, you will need to discuss with the research staff about the trial and fill out a separate informed consent form.

I don't want to be involved in a clinical trial. Should I still register?

Absolutely, we hope that you will still be willing to register, even if you don't want to take part in a trial. Your information may still be useful to researchers who are trying to learn more about patients with MCT8 deficiency.

What are my options if I do not want to be in the Registry?

You do not have to join this registry. Participation is voluntary. You do not need to participate in this Registry to remain or become a member of the MCT8 deficiency community. Your decision to participate in this registry or not will not affect your healthcare.

By signing this form you do not give away any legal rights or benefits to which you are otherwise entitled. If you do join, you can change your mind and withdraw from the registry at any time and request to remove any of your information that has not assigned yet to any specific study. You will not be able to remove any information that already has been assigned to a specific study. If you decide not to sign this form, there will not be any effect on your child`s regular health care, medical treatment or insurance benefits.

If you have any questions about the registry or need help, please contact us at:
mct8.deficiency@erasmusmc.nl