

Information for Children

“The International Dysferlinopathy Registry”

As you probably know, you have a condition called a dysferlinopathy. You have this condition because of changes in a part of your body that is called the dysferlin (*DYSF*) gene. These changes mean that certain cells of your muscles are damaged.

We would like to set up scientific research studies and clinical trials, so that we can learn more about your condition and help develop and test potential treatments that could help you keep your strength for as long as possible. We would like as many patients as possible, who – like you – have a dysferlinopathy, to participate in clinical trials and research studies for dysferlinopathy. In order to do this, we need to make a list of patients (called a registry) from around the world that have a dysferlinopathy. This registry means that patients worldwide can sign up to make it easier to plan these studies and clinical trials. With the help of this registry, we can also find out how many patients are living with your condition worldwide and if everybody gets the same care from their doctors.

In a registry, information is collected and stored on a computer. All patients in this registry have a dysferlinopathy, like you. For this registry – called the International Dysferlinopathy Registry – we want to know, for example, if you can walk or are using a wheelchair. We also ask for the exact changes in your dysferlin (*DYSF*) gene, the gene responsible for your disorder.

You can list all of your details online by filling out, with the help of your parents, the questionnaires found on the International Dysferlinopathy Registry website. Your personal information, such as name and address, will be securely stored on a computer in France and only the person in charge of the International Dysferlinopathy Registry will have access to these personal details. Your medical data will be stored on the same computer in France, and people who are planning research studies or clinical trials can view this part of your data, but these people will not know your name or where you live. You can always tell us if you do not want your information stored in the registry any longer. This will not affect your future care in any way.

If you are happy to have your information included in this registry, we would like to ask you (and your parent/guardian) to sign the *Registration Questionnaires I and II* to confirm this.

If you have any further questions about the International Dysferlinopathy Registry, please do not hesitate to ask your doctor or nurse. You can also contact the registry:

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Thank you for reading this patient information sheet.