



## INFORMED CONSENT FORM

Dear Patient [parent(s)/legal representative],

We invite you to take part in a patient registry for Rare Neuromuscular Diseases under the European Reference Network EURO-NMD: the EURO-NMD Registry. Participation is voluntary and requires your written consent as a legal basis to use your data [the data of your child/the patient]. Please read this information carefully and ask your medical doctor for an explanation if you have any questions.

### EUROPEAN REFERENCE NETWORK REGISTRIES

- European Reference Networks (ERNs) are networks of healthcare professionals for rare diseases across Europe working together to support patients with rare and complex diseases.
- EURO-NMD is the European Reference Network covering Neuromuscular Diseases, the large majority of which are simultaneously genetic conditions and Rare Diseases. The EURO-NMD Registry (<https://registry.ern-euro-nmd.eu>) is the platform that has been built to support the monitoring of the quality of care of patients treated within the participating health care providers of the ERN EURO-NMD, while providing precious input for a better understanding of these diseases, their diagnosis, management and treatment. Neuromuscular Diseases frequently cause chronic health problems, and many are life-threatening or present particular challenges for the quality of life of affected patients. They are infrequent and often require numerous resources and multidisciplinary teams to reach a correct diagnosis, management and treatment. The challenge to bring together sufficient data regarding these patients to launch research and clinical trials is fundamental for improving the condition of neuromuscular patients and can be achieved only by extensive data collection such as the one registries allow.
- To understand the course of a disease and investigate new diagnostic procedures and treatments in order to improve patient care, ERNs need databases (also known as “registries”) for research and knowledge development.
- To build such registries, data from many patients must be combined. We ask for your consent to include your data [the data of your child/ the patient] in the EURO-NMD Registry to perform monitoring of care quality and research, as described below, in accordance with national and European data protection laws and ethics guidelines<sup>1</sup>.
- Only the data required for such research will be recorded and may be shared with users as outlined below. Such data may include age, sex, the signs and symptoms of the disease, results of diagnostic procedures (e.g., laboratory test results, genetic information, imaging

<sup>1</sup> including the European General Data Protection Regulation (GDPR), Reg. (EU) 2016/679; the Declaration of Helsinki 2013; the International Ethical Guidelines for Biomedical Research Involving Human Subjects CIOMS-WHO (2016); the Oviedo Convention and its Additional Protocol on human rights and biomedicine, concerning biomedical research (2005); the [“standard contractual clauses for the transfer of personal data to third countries” \(EU\) 2021/914](#) and .... **<please include any other applicable law>**





studies), as well as therapeutic interventions and their long-term outcomes.

- Your data [your child/ the patient data] privacy will be secured as described below in this form. Only your doctor will be able to link your data to you. Therefore, the risk of re-identification by unauthorized persons is minimal.

## VALUE & BENEFITS

### HOW WILL THE DATA BE USED?

The data collected in this registry is used to improve the delivery of healthcare, including the diagnosis, treatment and prognosis of patients with Rare Neuromuscular Diseases.

The data may be used later on in order to perform research (academic, clinical, or industrial). Research is often carried out in collaboration with other researchers. By sharing data, more questions can be answered. Research is often carried out in collaboration with other researchers. By sharing data, more questions can be answered.

Only users authorised by the **Registry Data Access Committee** can use the data. This Committee is composed of qualified health professionals, patients' representatives as well as members with legal and ethical expertise. It ensures that the request for data use aligns with the purposes of the registry and its policy.

The Registry Data Access Committee may provide data access to **clinical researchers from within or outside the ERN EURO-NMD, patient organisations, and the pharmaceutical industry** in order to develop projects, policies or studies aimed to improve the delivery of healthcare for rare diseases. Also, registry data may be shared with **health authorities, policy makers and regulators** to inform their decisions on rare disease health policy and approval of medicines.

### Data use for commercial purposes

Companies might request access to data stored in the registry to perform research aimed to develop new therapies for your condition. For example, the registry can inform companies how many patients live with a certain disease and help find patients in clinical trials of new therapies.

Typically, the results of this research will become property of the company that may also use them for further **commercial purposes** and to patent. You [your child/ the patient] will not acquire any rights over these results, own them in any way, or be entitled to share any future financial benefit derived from this research.

You may choose if you want to allow the use of your data [your child/ the patient data] for commercial research.

### Data transfers outside the EU

Data without any personally identifiable information may also be forwarded to researchers working in countries outside the EU, where the General Data Protection Regulation (GDPR) does not apply. In this case, a written agreement will be set up to ensure that the data is processed in compliance with the GDPR.





You may choose if you want to allow the transfer of your data [your child/ the patient data] to non-EU countries to contribute to projects directly aligned with the aims of this registry within a framework compliant with GDPR.

### Future changes in data collection

To gain more insight on your condition [your child/ the patient condition] we may need additional data in the future. This information will be published on the registry website <https://registry.ern-euro-nmd.eu>.

Furthermore, we may request additional data from existing disease- or treatment-specific registries/databases, including other ERN registries. You may choose if you want to allow the linking of your data [your child/ the patient data] with additional data as described above.

### Re-contacting to participate in research projects

In the future, research projects on the diseases and conditions covered by this registry may be proposed. You may choose if you want to be re-contacted by your medical doctor [your child/ the patient medical doctor] to participate in such studies. If you agree to be contacted, you are free to refuse, without any prejudice, participation in the proposed studies after you have been fully informed. Your current care [the current care of your child/ the patient] will not change in any way if you choose not to give your consent.

## WHAT ARE THE BENEFITS?

While there is no direct benefit from participating in this registry, the knowledge about the disease will be improved. This may benefit you [your child/ the patient] and other patients suffering from the same disease by improving healthcare and quality of life.

The participants may benefit by facilitated access to clinical studies aimed to prevent and treat the disease.

### Communication of research results

The results of the research will be communicated through the registry website (the information about projects given access to registry data is publicly available on the registry website), conferences or publications in scientific journals where personal data are not provided. The privacy of your data [your child/ the patient data] will always be protected as described below.

## PROTECTION

### WHAT ARE THE RIGHTS OF THE REGISTRY PARTICIPANT?

- The processing of your personal data [your child/ the patient personal data] is necessary for the conduct of the registry and is based on the consent
- You decide whether to participate in the registry [let your child/ the patient participate in the registry]. Please take as much time as you need to make this decision. You do not have to sign anything. You can decline participation without giving any reasons. You [your child/ the patient] will receive the same treatment irrespective of whether or not you agree to participate in this registry. Even if you agree to participate in the registry, you may at any time oppose





the processing of your personal data [your child/the patient personal data] for the purpose of carrying out research. In this case, no additional information about you [your child/ the patient] will be collected.

- You have the right to give or withhold your consent at any time. If you consent today, you may modify or withdraw your consent later, without any prejudice. Your doctor will explain how your consent can be modified and how the data can be removed from the registry if you wish so. Please be informed that, to guarantee the validity of any research performed, data already processed cannot be deleted. However, this data will not be used in new research projects after withdrawal.
- You can also request the limitation of the processing of your personal data [your child/ the patient data] (i.e. ask the promoter to temporarily freeze the use of your personal data [your child/ the patient data]).
- You also have the right to portability of your data [your child/ the patient data] (the right to portability gives you the possibility to recover part of your data [your child/ the patient data] in a machine-readable format).
- You are entitled to receive further information about the purposes for which your data [your child/ the patient data] will be processed and who will have access to it. You can also request to access your data [your child/ the patient data] at any time.
- The hospital where you are treated [your child/ the patient is treated] is the local controller responsible for the **local protection** of confidential patient data. If you have any concerns about the way in which your data [your child/ the patient data] is processed, you would like more information or to exercise your rights, you may contact your medical doctor [your child/ the patient medical doctor]. In case of difficulty, you can contact the Data Protection Officer of your hospital [your child/ the patient hospital], or you may raise a complaint to the relevant data protection authority. You can find contact details of the local Data Protection Officers at the registry website (<https://registry.ern-euro-nmd.eu>). They have the duty to ensure the data is processed safely and to notify you if a breach of data security occurs. Any inquiries should be addressed by the Data Protection Officer within 30 days.
- For all data submitted to the **central registry database**, the Assistance Public Hôpitaux de Paris (AP-HP), Coordinator of the European Reference Network for Rare Neuromuscular Diseases (ERN EURO-NMD), and its principal investigator, Dr. Teresinha EVANGELISTA, Neurologist at AP-HP, is responsible for the storage and use of transferred data: The AP-HP Data Protection Officer may be contacted at : [protection.donnees.dsi@aphp.fr](mailto:protection.donnees.dsi@aphp.fr)

## HOW WILL DATA BE SECURED?

- Participation in the registry will be kept strictly confidential and all information will be handled through very secure electronic systems. As the registry involves collecting information from many centres, the system will be password protected and only persons specifically involved with the registry will have access.
- The registry users and administrators will not be able to contact you because your name, address and hospital number [your child/ the patient name, address and hospital number] will not be recorded. All your data [your child/ the patient data] will be pseudonymised before being stored in the registry. This means that all identifiers that relate to you [your child/ the





patient] will be removed and replaced by a pseudonym<sup>2</sup>. Only your medical doctor [your child/ the patient medical doctor] can link the pseudonym to you [your child/ the patient]. Therefore, the risk of re-identification by unauthorized persons is minimal.

- In all publications emerging from the registry, it will be ensured that it is not possible to identify an individual patient, e.g., by providing data in tables or presenting age categories rather than the real age.
- A pseudonymisation service will be used for this purpose. It allows to identify duplicate registration of patients, linkage between registries and other data resources, keep data protected and preserve the possibility of re-contacting by the medical doctor in charge
- The registry data will be stored on a secure REDCap server physically located at the Clinical Trials Unit of the University Medical Center Freiburg that contain all necessary security facilities for at least 10 years.

## COULD PARTICIPATION IN THE REGISTRY CAUSE ANY HARMS?

- Participating in this observational registry will not cause any health risks
- Even though the registry has processes in place to ensure your personal information is protected, there is a remote risk the data could be matched with information you have already authorized in publicly available databases such as ancestry websites or public rare disease registries with identifiable information. To minimize this risk, researchers asking for access to registry data will confirm in writing not to try to identify you by any means, applying their duty of professional secret.

## ADDITIONAL INFORMATION

### Costs

Participation in this registry will not entail any costs for you.

### Insurance

<please include information about insurance taken for the registry activities if applicable, as requested by some Ethics Committees – otherwise, please delete this paragraph>

### Ethics Committee Approval

This Informed Consent Form has been reviewed and approved under the number <Ethics Committee/ IRB number> by [name of the (local) Ethics Committee/IRB

If you have any other question about the registry, please contact the central registry office at [registry@ern-euro-nmd.eu](mailto:registry@ern-euro-nmd.eu)

<sup>2</sup> A pseudonym is a sequence of letters and numbers that replaces all identifiers that relate to a patient; the data of the patient is then called “pseudonymised data”. These identifiers can only be retrieved, from the pseudonym, by the authorised health care professionals enrolling the patient in the registry.





## INFORMED CONSENT

Patient First and Last Name:.....

Date of Birth (dd/mm/yyyy):...../...../..... ID number:.....

I have read the information sheet about the EURO-NMD Registry.

I have been given the time and opportunity to ask questions about the objectives of the registry and the use of my data [my child/the patient data] and that I have solved all my doubts with the medical doctor.

I understand that my participation is voluntary and that I can withdraw the consent at any time without the need of justification and without affecting my future medical care [the future care of my child/the patient].

I approve that my data [my child/the patient data] will be stored in the EURO-NMD Registry, used for non-profit purposes and shared with approved users to improve the delivery of healthcare as described above.

I consent to the processing of my pseudonymized data [my child/the patient pseudonymized data] for the purposes described above.

**The following consent conditions are optional.** Please indicate your preferences by writing your initials in the relevant box. If you leave the boxes **EMPTY**, we assume you **DO NOT AGREE** to the statements:

YES	NO	
<input type="checkbox"/>	<input type="checkbox"/>	<b>I CONSENT</b> that my pseudonymized data [my child/the patient pseudonymized data] may also be <b>used to support commercial projects</b> aimed to improve healthcare.
<input type="checkbox"/>	<input type="checkbox"/>	<b>I CONSENT</b> that my pseudonymized data [my child/the patient pseudonymized data] <b>may be transferred to non-EU countries, in compliance with GDPR</b> , to support projects aimed to improve healthcare.
<input type="checkbox"/>	<input type="checkbox"/>	<b>I CONSENT</b> that my pseudonymized data [my child/the patient pseudonymized data] may be <b>linked to existing databases/registries</b> to improve healthcare.
<input type="checkbox"/>	<input type="checkbox"/>	<b>I WOULD LIKE TO BE CONTACTED</b> by my medical doctor [my child/the patient medical doctor] about any <b>research project and/or clinical study related to my condition</b> [my child/the patient condition].





**EURO-NMD  
Registry Hub**

European Reference Network  
for neuromuscular diseases

**I CONSENT** to provide my email address and be contacted to complete questionnaires about my health [the health of my child/the patient] and receive news and updates about the registry.

E-mail address:.....

**PATIENT [PARENT/LEGAL GUARDIAN]**

Date and Signature:

**MEDICAL DOCTOR / AUTHORISED WITNESS**

Full name:

Position:

Date and Signature:

**Please keep one copy of this Informed Consent Form in case records and hand one copy to the person who has signed this form.**



*This document is part of the project 'EURO-NMD Registry' co-financed by the European Commission under the Third Health Program (grant agreement 101055286). The EURO-NMD Registry has also received funding for 5 months from the EU4health program (grant agreement 101085084) and will continue to be funded for the 2023-2027 period under the call EU4H-2023-ERN-IBA.*