

Swiss Registry for Neuromuscular Disorders

(Swiss-Reg-NMD)

In this text, the male form is used for reasons of simplicity. The female form is of course always included.

Study information for parents

Dear parents

We would like to provide you with some information about our research project. Please read this information carefully. Do not hesitate to discuss your questions with your child's doctor or to contact us for additional information.

What is the Swiss Registry for Neuromuscular Disorders?

The Swiss Registry for Neuromuscular Disorders collects medical information in a database from children, adolescents and adults with diseases that affect the muscles or nerves.

The registry was founded in 2008 in Lausanne. Since 2017 it is located at the Institute for Social and Preventive Medicine of the University of Bern. PD Dr. med. Andrea Klein and Prof. Dr. med. Claudia Kuehni lead the registry in collaboration with specialised clinicians from all major hospitals in Switzerland. The competent ethics committee (EC Bern) has reviewed and approved this registry.

Why is information on neuromuscular diseases being collected?

In past years, significant advancements have been made in the area of research on neuromuscular diseases. First drugs have been tested and approved for the treatment of certain diseases. New drugs are being continuously developed and tested. Because neuromuscular disorders are very rare diseases, drug studies need to be conducted simultaneously in several centres in Switzerland and abroad. The Swiss Registry for Neuromuscular Disorders was created to facilitate access for Swiss patients to drug studies. The registry helps us quickly find patients eligible for a study, inform them, and enable them to participate. Participation in such studies is always voluntary.

During your child's medical treatment, the doctor documents how and why the diagnosis was made, how your child is doing, how he/she is developing, and which therapies are used. These data are in your child's medical records and can be very important for the research. The national evaluation of these data helps us to obtain a comprehensive picture of the care and well-being of patients with neuromuscular diseases in Switzerland. Thus, it is important for us to be able to evaluate information from as many affected persons as possible.

For this reason, we would like to invite you to enrol your child in the Swiss Registry for Neuromuscular Disorders and allow us to collect your child's medical information.

What are the objectives?

The main objective of the Swiss Registry for Neuromuscular Disorders is to facilitate access for Swiss patients to drug studies. Thanks to the data collected, we check whether your child meets the inclusion criteria of an upcoming study. If this is the case, we will inform you or your child's attending doctor about the study. In this way, you can be referred to the study centre, if you wish. Participation in such studies is always voluntary.

The registry also aims to examine how often neuromuscular diseases occur, how the diseases progress

over time, how they are treated, and how the symptoms and the various treatments affect patient's quality of life. In this way, patients' well-being can be improved in the future. We would like to know, for example, which support in school facilitates integration into the world of work and which needs people with neuromuscular diseases and their families have. Through the central collection of data, the treatment can additionally be compared between centres, standardised, and improved over the long term.

Which persons will be included?

All persons with a neuromuscular disease who are treated in Switzerland can participate in the registry.

Which information is recorded?

No examinations or treatments are specially performed for this registry. We will only document in the database what has already been examined in or prescribed to your child in the hospital or medical practice.

Disease-related data: This information is documented in your child's medical records. It contains data on the diagnosis, the course of disease (such as motor function, heart and lung function), symptoms (such as pain) and treatments (such as physiotherapy, surgery, drugs). If you and your child agree, we will collect data on these topics.

Administrative data: In addition, we collect administrative data, such as name, address, email and date of birth, as well as name, dates of birth and occupations of both parents and address of the paediatrician. This is necessary so that we can contact you if your child participates in a study. In this way, at a later point, we can allocate new information to the right person and send questionnaires to the families.

Questionnaires: From time to time, we would like to send you and your child a questionnaire on aspects that you can answer better than your child's doctor. The questions may concern, for example, quality of life, symptoms, frequency of therapies, school, or families' concerns. You may of course decide each time whether or not you wish to answer the questionnaire.

Linking with other data or databases: The Swiss Registry for Neuromuscular Disorders is linked with national and international registries, for example with the Swiss Rare Disease Registry (SRDR, www.raredisease.ch) and with TREAT-NMD (www.treat-nmd.org). We determine address changes by enquiring with municipalities or Swiss Post. In addition, the registry can use existing statistical data, such as information from the registry of births, of cause of deaths or hospital statistics from the Swiss Federal Statistical Office, or therapy costs from health or disability insurance. As a result, many scientific questions can be investigated without having to repeatedly ask patients for further information.

Additional information: Since early education and school have a significant influence on children's development, we would also like to collect data from school reports and assessment reports.

Invitations to participate in in-depth studies: If an in-depth study on your child's disease takes place in Switzerland in the next few years, we may invite you to participate in it, for example, to conduct special examinations. Participation in such studies is always voluntary.

What benefits do participants have?

Patients whose data were collected can be quickly and reliably identified and informed if they are eligible for a drug study. Participation in such studies is always voluntary.

Your child's participation in the Swiss Registry for Neuromuscular Disorders also helps to better understand the course, symptoms and treatment of patients with neuromuscular diseases in Switzerland. Investigational methods, treatment, preventive measures and the medical care of people with neuromuscular diseases in Switzerland can be improved only if enough information is available for the research. You may also request an extract from your child's data from us at any time, for example, when switching to a new doctor.

What happens to the results?

The Swiss Registry of Neuromuscular Disorders informs the participating doctors of current studies or research results and they then discuss how they wish to implement these results. This may improve your child's treatment and the treatment of children affected in the future. We will also publish the results in scientific publications (journals for doctors and therapists) and on the website of the registry. We also write independent reports for patient organizations, responsible bodies of the Swiss healthcare system (e.g., the Federal Office of Public Health) and the pharmaceutical industry. Reports inform about our results e.g., about the effect and side effects of new approved therapies or about the number of patients who receive or are eligible for a new therapy. Reports and publications never contain personal information and in no case is it possible to identify individual persons.

Are the data handled confidentially?

All information is handled strictly confidentially at all times. Only employees of the Swiss Registry for Neuromuscular Disorders or researchers who absolutely require the data for their work may work with the data. The employees of the Bern Cantonal Ethics Committee and the cantonal or federal data privacy officer may inspect the confidential data in order to carry out controls. All of these persons are bound to a duty of confidentiality. The data privacy measures are adapted in each case to the most current requirements. All data are always stored such that they are inaccessible to third parties. Destruction of the data is not planned.

For research purposes only coded data (without names or identifying information) is evaluated.

Will the data be forwarded?

Extracts of the data may be forwarded in coded form (without names or identifying information) for example to the international registry for the planning of studies or for research purposes. The management of the Swiss Registry for Neuromuscular Disorders reviews every request for use of the data for research purposes. If the planned research project meets all legal requirements and meets the objectives of the Swiss Registry for Neuromuscular Disorders, the data may be provided in coded form (without names or identifying information) for this research project.

This also applies to international studies. Many important questions can be answered only through international collaboration. A good example of this is the international registry of TREAT-NMD.

Can we refuse consent or withdraw it later?

Participation in the Swiss Registry for Neuromuscular Disorders is voluntary. You can refuse it at any time or withdraw it without stating a reason. You alone can decide whether you provide your child's data. A refusal now or a withdrawal later will not have any consequences for your child's medical care. If you do not consent, only minimal information without names and addresses will be collected.

Obligations

You and your child do not enter into any commitments with your participation.

Risks

No additional examinations or treatments will be performed for the study.

Is participation compensated?

If your child takes part in this project, he/she will not receive any compensation. Your child, you and the health insurance company should not incur any project-specific costs.

How is the registry funded?

The Swiss Registry for Neuromuscular Disorders is funded by patient organisations and private foundations which want to improve the care of patients with neuromuscular diseases. Also

pharmaceutical companies who are active in the field of neuromuscular diseases can support the registry. The registry is always independent in its activities. The participating doctors, hospitals and clinics also make an important personal contribution through their work.

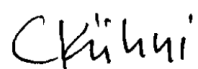
Do you have additional questions?

If you have any questions, please contact your child's attending doctor or the coordinator of the registry directly:

Swiss Registry for Neuromuscular Disorders
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University of Bern
Mittelstrasse 43
3012 Bern
Telephone: +41 31 684 68 89
Email: swiss-reg-nmd.ispm@unibe.ch

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Hospital/medical practice

Prof. Dr. med. Claudia Kühni
Head of Swiss-Reg-NMD
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University of Bern



Swiss Registry for Neuromuscular Disorders (Swiss-Reg-NMD)

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Informed consent form for participation in the Swiss-Reg-NMD

- Please read this form carefully.
- Please ask if you do not understand something or need more information.

Number of the research project (at the competent ethics committee)	2018-00289
Head of the research project Responsible institution	Prof. Dr. med. Claudia Kuehni Institute for Social and Preventive Medicine University of Bern
Participant	
Surname and first name <i>(printed characters)</i>	
Date of birth	
	<input type="checkbox"/> female <input type="checkbox"/> male

We confirm, as the parents of the abovementioned child, that:

- We have received, read and understood the written information (version 2.1. – 25.07.2022). Our questions about the Swiss Registry for Neuromuscular Disorders (Swiss-Reg-NMD) have been answered to our satisfaction. We may keep the written information.
- We were informed that registration in the Swiss-Reg-NMD and participation in any resultant studies is voluntary.
- We had enough time to make a decision.
- We know that we and our child may withdraw this consent any time, without stating any reasons. We also know that this will not result in any disadvantages with regard to the further treatment and care of our child. The child’s data will then be anonymised in the Swiss-Reg-NMD.

With our signature, we declare our consent:

- for the data and medical documents regarding our child’s neuromuscular disease to be forwarded by all attending doctors and their employees in medical practices and hospitals to the Swiss-Reg-NMD.
- for the data to be recorded in the Swiss-Reg-NMD and used for the research purposes described.
- for the data to be forwarded to third parties for research purposes, also abroad and in particular to the international registry of TREAT-NMD. The data will be forwarded only in encoded form and without names.
- for our child’s data and medical documents to be stored for an indefinite period if this needed for the research.

- for the data of our child and the parents to be supplemented from other sources (medical registries, data from the Swiss Federal Statistical Office, disability insurance, population registers, school reports, assessment reports).
- for the Swiss-Reg-NMD to contact us or our child in order to collect data using questionnaires or interviews on current health status or invite us to participate in a drug study or another in-depth study. With each inquiry, we may freely decide whether or not to participate.
- for the general practitioner, other attending doctors and therapists to be contacted for enquiries.
- for the responsible specialists of the project management of the study and the ethics committee responsible for this project to inspect the child's uncoded data for inspection and monitoring purposes, however in strict observance of confidentiality.
- for the names, dates of birth, address and occupations of the parents to be recorded.
- that our child agreed to participate and/or that no signs of opposition to participation have been indicated.

For children and adolescents <18 years

Place, date	Surname and first name of the mother
	Signature
Place, date	Surname and first name of the father
	Signature

Whenever possible, both parents should sign. One signature is sufficient for the registration.

Physician confirmation: I hereby confirm that I explained to this participant and the legal representative the nature, significance, and scope of the study. I confirm that I will fulfil all obligations in connection with this study in accordance with applicable law. If, at any time, I learn about issues which could influence the participant's willingness to participate in the study, I will inform him/her and his/her legal representative of this immediately.

Place, date	Surname and first name of the physician providing information
	Signature