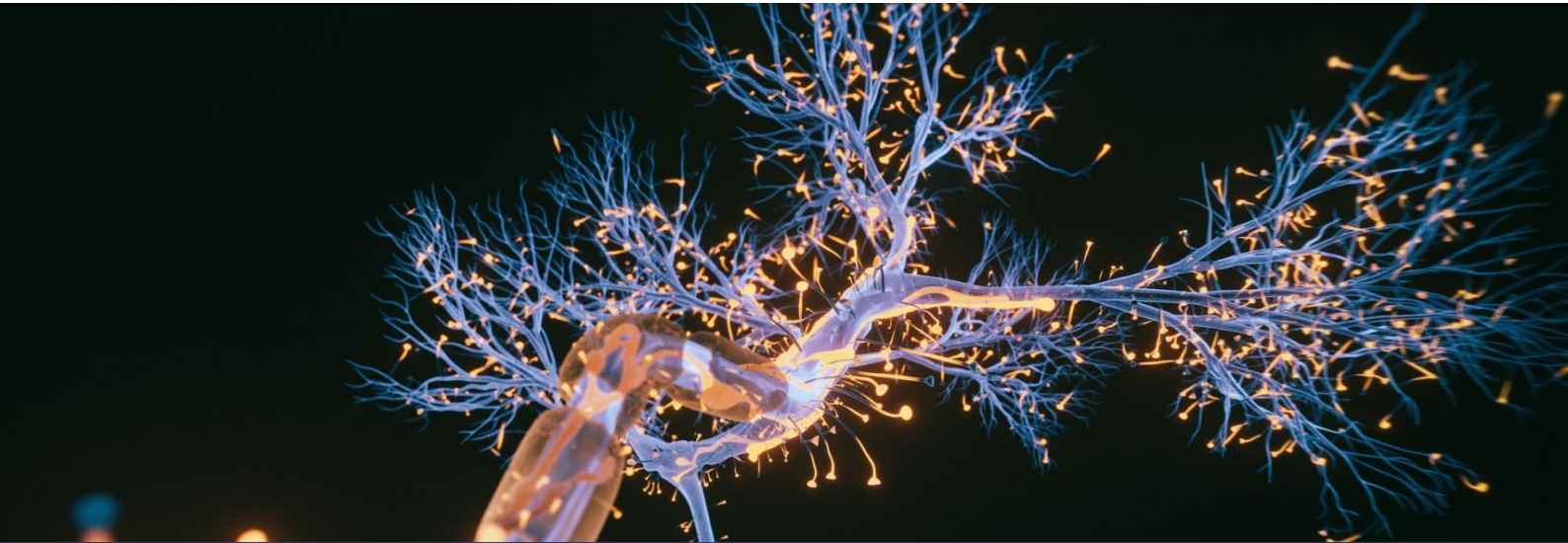




Annual Report 2025

Swiss Registry
for Neuromuscular Disorders

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Swiss Registry for Neuromuscular Disorders

Annual Report 2025

Bern, September 2026

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*On behalf of the **Swiss-Reg-NMD** group*



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Message from the clinical lead

Im vergangenen Jahr konnte das Register mit zwei wissenschaftlichen Artikeln, einem weiteren Evaluationsbericht zur Therapie-Wirksamkeit sowie der Bearbeitung von rund 30 Datenanfragen für unterschiedlichste Zwecke (z. B. Lehre oder Forschung) erneut einen wesentlichen Beitrag als nationale Forschungsplattform leisten.

Wichtige Meilensteine waren zudem der erfolgreiche Aufbau der Infrastruktur zur Erfassung von Daten zur Pompe-Krankheit sowie die Aufnahme von Swiss-Reg-NMD als offizielles Mitglied im KOSEK anerkannten Netzwerk *Myosuisse*.

Diese Erfolge wären ohne das Engagement aller Patienten, Fachkräfte des Gesundheitswesens, Forschenden und Partnerorganisationen nicht möglich gewesen, und wir danken allen von ganzem Herzen für ihren anhaltenden Einsatz.

L'année dernière, le registre a une nouvelle fois apporté une contribution significative en tant que plateforme nationale de recherche, grâce à la publication de deux articles scientifiques, à la publication d'un nouveau rapport d'évaluation sur l'efficacité thérapeutique et au traitement d'une trentaine de demandes de données à des fins très diverses (enseignement, recherche, etc.).

Parmi les étapes importantes, citons également la mise en place réussie de l'infrastructure de collecte de données sur la maladie de Pompe, ainsi que l'adhésion de Swiss-Reg-NMD en tant que membre officiel du réseau *Myosuisse*, reconnu par la KOSEK.

Ces succès n'auraient pas été possibles sans l'engagement de tous les patients, des professionnels de santé, des chercheurs et des organisations partenaires. Nous les remercions du fond du cœur pour leur dévouement sans faille.

Lo scorso anno, il Registro ha potuto fornire nuovamente un contributo significativo in qualità di piattaforma nazionale di ricerca, grazie alla pubblicazione di due articoli scientifici, a un ulteriore rapporto di valutazione sull'efficacia terapeutica e all'elaborazione di circa 30 richieste di dati per le finalità più diverse (ad esempio, didattica o ricerca).

Traguardi importanti sono stati inoltre la riuscita realizzazione dell'infrastruttura per la raccolta di dati sulla malattia di Pompe e l'ammissione di Swiss-Reg-NMD quale membro ufficiale della rete *Myosuisse*, riconosciuta da KOSEK.

Questi successi non sarebbero stati possibili senza l'impegno di tutti i pazienti, degli operatori sanitari, dei ricercatori e delle organizzazioni partner: a tutti va il nostro più sentito ringraziamento per la loro costante dedizione.



Clinical Lead

Prof. Dr. med. Andrea Klein
Neuropediatrician
Interspital Bern



Clinical Lead a.i.

Dr. med. David Jacquier
Pediatrician
CHUV



Vice Clinical Lead a.i.

Prof. Dr. med. Olivier Scheidegger
Neurologist
Inselspital Bern

Spotlight 2025

In 2025 the registry's work was guided –as in the past –by our long-standing goal to improve care, well-being, and inclusion of patients with neuromuscular disorders (NMD) in Switzerland.

By end of 2025, the registry counts 499 registered individuals with 7 different NMDs. For 77% of them records from 2025 are available. Up-to-date data forms the basis of the registry, enabling the generation of representative results, to tackle current and future research questions, greater participation in international projects and adds weight to the voices of those affected. For 2025 we can highlight the following:

Inclusion of Pompe Disease

During 2025 we developed a database to collect medical data of patients with Pompe disease who live or are being treated in Switzerland. The database replaces the former Swiss Pompe registry managed by Sanofi-Aventis Schweiz AG.

Swiss-Reg-NMD is a Core Member of Myosuisse

Myosuisse¹ is the Swiss network of experts and organizations dedicated to the care and well-being of people living with a neuromuscular disorder recognized by KOSEK². At the general assembly in August 2025 in Bern the networks' governance structure was approved. Thereby the Swiss-Reg-NMD has become an official core member of Myosuisse.

Peer-reviewed Articles

During 2025 two scientific articles have been published in peer-reviewed journals. One about the long-term (>4 years) effect of treatment with nusinersen in the Swiss cohort of patients with spinal muscular atrophy. The other about the education and participation of children and adolescents with Duchenne muscular dystrophy in Switzerland.

Strategic Meetings with Pharmaceutical Companies

Registration of people with rare diseases and the assessment of their medical history are beneficial to healthcare professionals, pharmaceutical companies, and authorities. Currently, there is no legal basis for running disease-specific national registries, and consequently no funding available from authorities. Particularly in the case of rare diseases, patient organizations and medical societies are usually small and cannot cover the running costs of such a registry. Therefore, the registry's management has reached out to partners from the pharmaceutical industry to discuss the financial situation of the registry and basic funding opportunities.

Rare Disease Legislation

During the last year a draft has been developed and published by the Federal Office of Public Health (FOPH) which aims to regulate the measures to tackle rare diseases (*Bundesgesetz über Massnahmen zur Bekämpfung seltener Krankheiten*³). Key features of the legislation are a rare disease registry, financial support for specialized care delivery structures, and financial support for information and awareness activities. The Swiss-Reg-NMD took the opportunity to submit a statement during the consultation procedure.

New Guidelines out now

Well defined data items are key for the collection of reliable and reusable data from different physicians and centers. The registry's guidelines for data collection and data entry have been thoroughly revised during the last two years and were finally distributed to all data providers of Swiss-Reg-NMD in July 2025.

¹ <https://myosuisse.org/>

² Kosek = Nationale Koordination Seltene Krankheiten; <https://www.kosekschweiz.ch/>

³ https://www.fedlex.admin.ch/filestore/fedlex.data.admin.ch/eli/dl/proj/2024/78/cons_1/doc_1/de/pdf-a/fedlex-data-admin-ch-eli-dl-proj-2024-78-cons_1-doc_1-de-pdf-a.pdf

The Registry

The Swiss Registry for Neuromuscular Disorders (Swiss-Reg-NMD or ‘registry’) aims to improve the care, well-being, and inclusion of patients living with a neuromuscular disorder. We collect relevant health data of patients with a neuromuscular disorder living or treated in Switzerland. The registry provides the basis for a variety of research projects, post-marketing surveillance endeavors, and national and international networking. Table 1 is an overview of the aims and main instruments of Swiss-Reg-NMD.

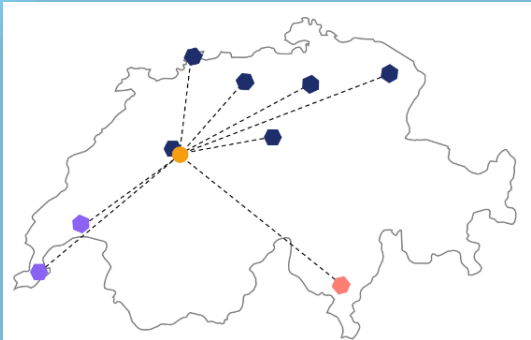
Goal			
Improve care, well-being, and inclusion of individuals with neuromuscular disorders			
Aims	Research & post-marketing surveillance	Epidemiology	Networking
Instruments	Analyses of disease progression and treatment effectiveness Research on quality of life Support for clinical trials (e.g. invite patients) Health system evaluations	Monitoring of incidence, prevalence, and care data	Data repository for research Internationally harmonized datasets Platform for knowledge exchange National & international collaborations
Basis	Collection of real-world medical follow-up data from/by all KOSEK-recognized reference centers for neuromuscular disorders (map) as well as further regional hospitals and local practices. Registration of patients with an NMD living or being treated in Switzerland. 		

Table 1: Overview of the aims and main instruments of the Swiss Registry for Neuromuscular Disorders. KOSEK = Nationale Koordination Seltene Krankheiten; NMD = neuromuscular disorders.

Legal notice: Since 2018 the registry operates under the authorization of the Bern Cantonal Ethics Commission (BASEC #2018-00289). Data is collected from patients who have given written consent to participate in the registry study.

Organization

Swiss-Reg-NMD is managed by the clinical lead and the executive office. The clinical lead and a legal representative share the overall responsibility for the registry. Since August 2025 David Jacquier and Olivier Scheidegger act as interim clinical lead and vice clinical lead respectively due to a longer absence of Andrea Klein. The registry has a steering board that meets twice a year, once in person and once online. The board defines among other things the objectives, the research questions and the dataset of the Swiss-Reg-NMD and consists of pediatric neurologists, neurologists and a pulmonologist from different neuromuscular centers in Switzerland, and a representative of a patient organization. The registry works closely together with the participating centers. The organizational structure of Swiss-Reg-NMD is shown in Table 2.

Legal Representative / Sponsor		
Claudia Kuehni, Prof. MD	Legal representative	ISPM, University of Bern
Steering Board		
Andrea Klein, Prof. MD	Clinical Lead, Pediatric Neurologist	Inselspital Bern
David Jacquier, MD	Vice Clinical Lead, Pediatrician	CHUV Lausanne
Paolo Ripellino, PD MD	Neurologist	EOC Lugano
Georg Stettner, PD MD	Pediatric Neurologist	Kinderspital Zürich
Olivier Scheidegger, Prof. MD	Neurologist	Inselspital Bern
Thomas Hundsberger, Prof. MD	Neurologist	KSSG
Bettina Schreiner, PD MD	Neurologist	Universitätsspital Zürich
Esther Irene Schwarz, Prof. MD	Pulmonologist	Universitätsspital Zürich
Robert Palm	Patient representative, President Progena	Progena Schweiz
Executive Office		
Andrea Mathis, MSc	Project manager	ISPM, University of Bern
Dominique Baumann, PhD	Project manager	ISPM, University of Bern
Anne Tscherter, PD PhD	Project manager	ISPM, University of Bern
Simona Pfister, PhD	Project manager	ISPM, University of Bern
Lorena Hulliger, MSc	Project manager	ISPM, University of Bern
Anna Born, BSc	Data manager	ISPM, University of Bern
Advisors		
Franziska Joncourt, PhD	Genetic curator	Inselspital Bern
Christiane Zweier, Prof. MD	Genetic curator	Inselspital Bern
Katarzyna Pospiesznyi, MD	Expert for cranial MRI	Inselspital Bern
Richard McKinley, PD PhD	Expert for artificial intelligence	Inselspital Bern
Participating centres		
Aarau, Basel, Bern, Geneva, Lausanne, Luzern, St. Gallen, Ticino, Zürich, and private practices.		

Table 2: People and clinical centers involved at Swiss-Reg-NMD. ISPM = Institute of Social and Preventive Medicine, EOC = Ente Ospedaliero Cantonale, CHUV = Centre hospitalier universitaire Vaudois, KSSG = Kantonsspital St. Gallen.

Steering board meeting in Bern

The annual in-person steering board meeting took place in April 2025 at the ISPM, University of Bern. The meeting focused on the priorities regarding projects and the registries long-term funding. Once more we had fruitful discussions with our steering board members and were able to decide on measures to move Swiss-Reg-NMD forward.

The Registry in Numbers

All people with a neuromuscular disorder who live or are treated in Switzerland can participate in the registry. By the end of 2025, the registry counted 499 live patients with a confirmed diagnosis of one of the following diseases:

- Spinal Muscular Atrophy (SMA)*
- Dystrophinopathies: Duchenne-, Becker- and Intermediate muscular dystrophy (DMD/BMD/IMD)*
- LAMA2-related congenital muscular dystrophy (LAMA2-CMD) (also called Merosin-negative congenital muscular dystrophy (MDC1A))*
- Pompe disease (late onset) (LOPD)*
- Collagen VI-related dystrophy (COL6-RD)
- Limb-girdle muscular dystrophy (LGMD)
- Friedreich Ataxia (FA)

Of patients with one of the first four (*) diseases, we regularly collect structured medical data using a case report form. In the following we present key numbers of the registry cohort by 31.12.2025 (includes data reported to the registry until May 2026).

Patients

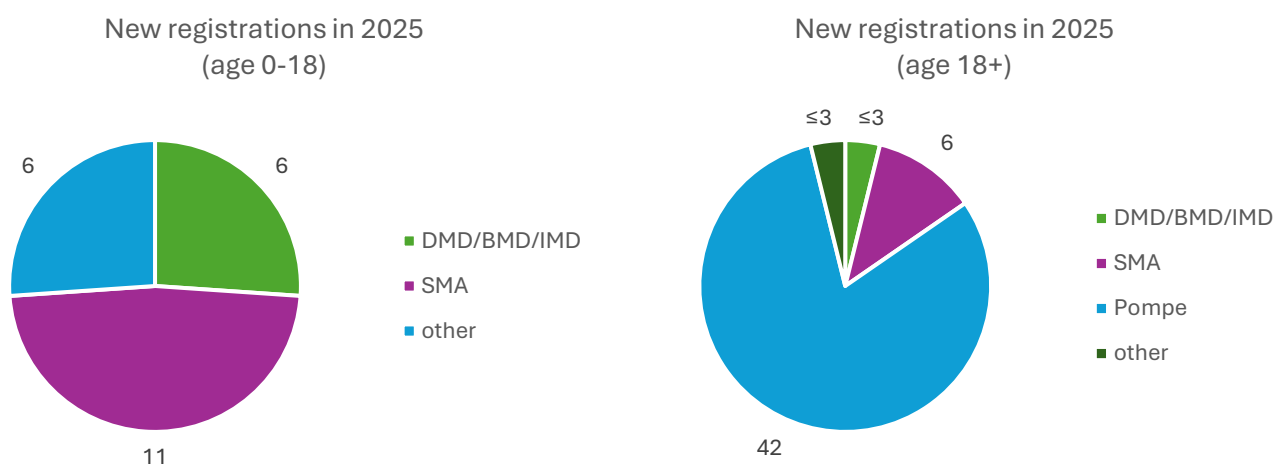


Figure 1: Number of new registrations in 2025 grouped by diagnosis and age. ‘Age’ refers to age by end of 2025. Total 75 patients have been newly registered in 2025. ‘Other’ includes Friedreich ataxia, collagen VI-related dystrophy and limb-girdle muscular dystrophy.

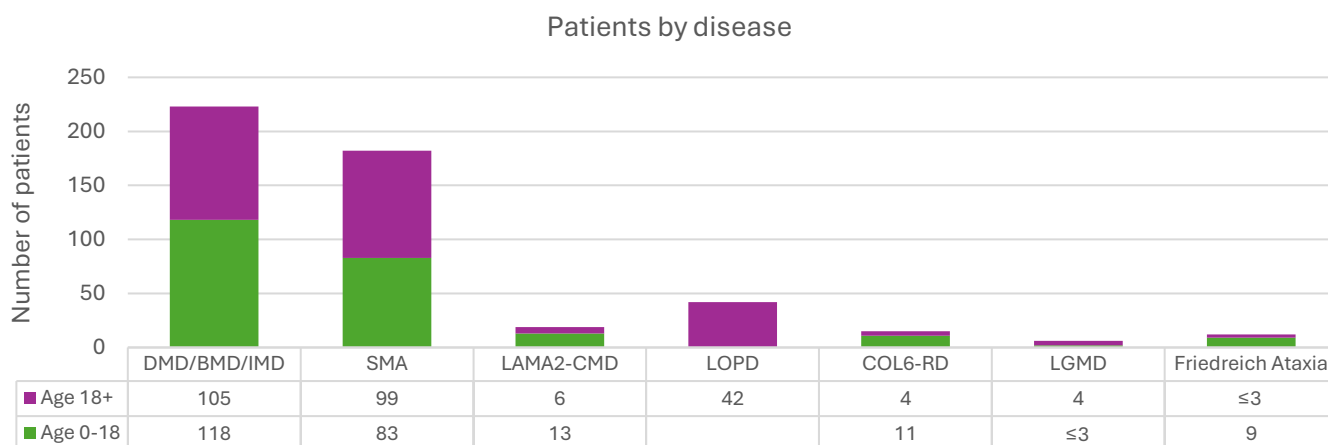


Figure 2: Registered patients by disease and age. ‘Age’ refers to age as of the end of 2025. All registered patients who have not been reported as deceased, moved abroad or lost to follow-up by 31.12.2025 are included.

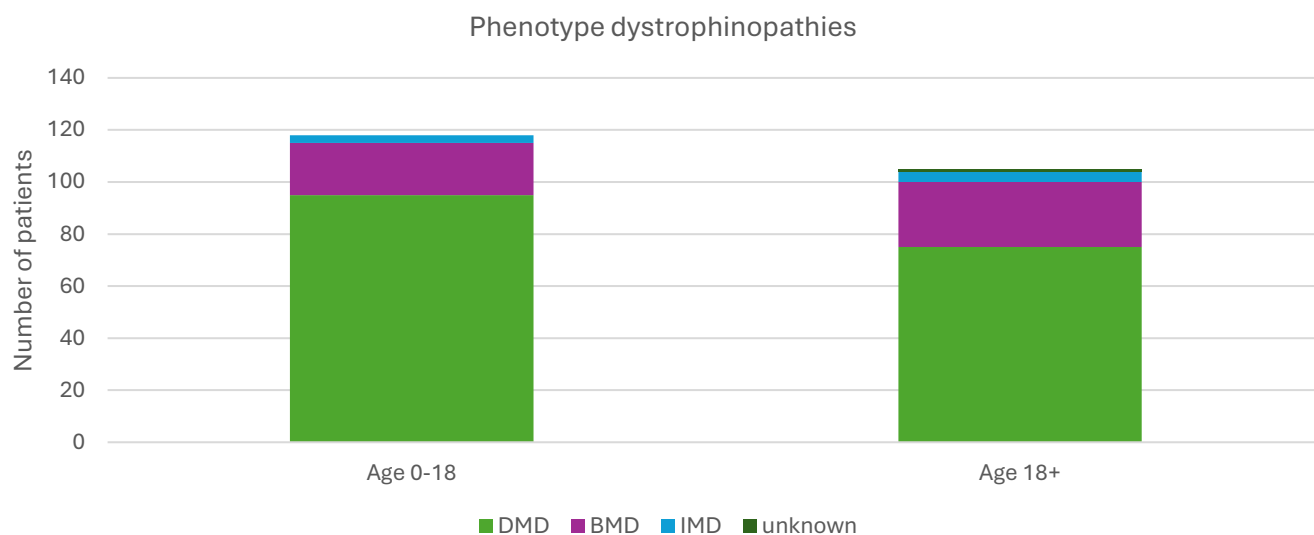


Figure 3: Patients registered with confirmed dystrophinopathy by phenotype and age. ‘Age’ refers to age at the end of 2025. All registered patients who have not been reported as deceased, moved abroad or lost to follow-up by 31.12.2025 are included.

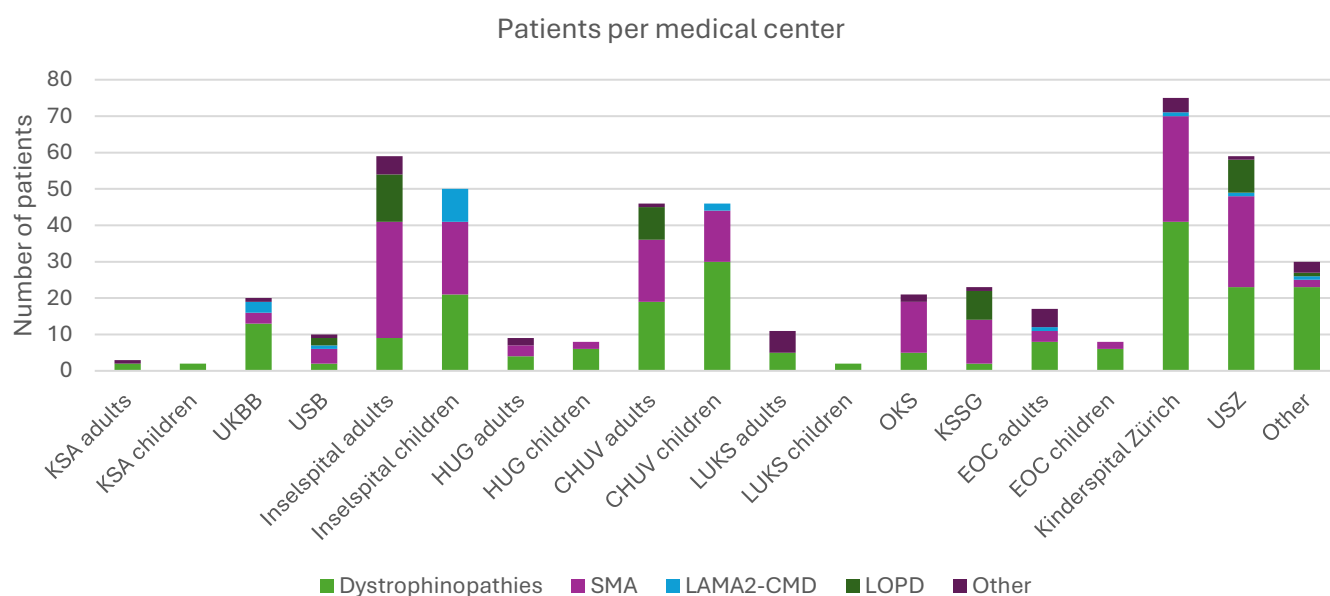


Figure 4: Registered patients per main medical center. All registered patients who have not been reported as deceased, moved abroad or lost to follow-up by 31.12.2025 are included. KSA = Kantonsspital Aarau, UKBB = Universitäts-Kinderspital beider Basel, USB = Universitätsspital Basel, HUG = Hôpitaux universitaires de Genève, CHUV = Centre hospitalier universitaire Vaudois, LUKS = Luzerner Kantonsspital, OKS = Ostschweizer Kinderspital, KSSG = Kantonsspital St. Gallen, EOC = Ente Ospedaliero Cantonale, USZ = Universitätsspital Zürich, other = private practices or residential home.

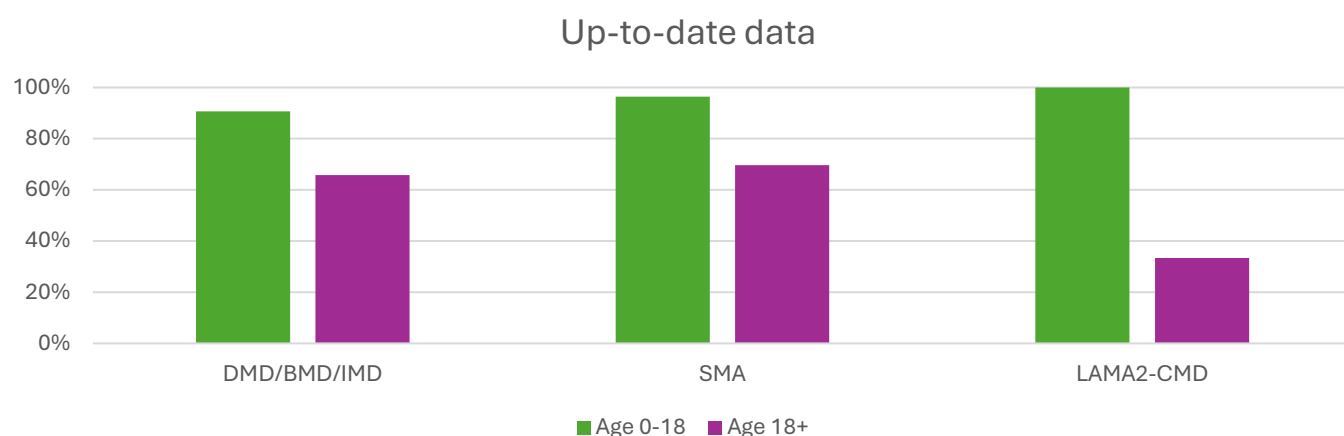


Figure 5: Percentage of patients of whom the registry received data for 2025 by disease and age. ‘Age’ refers to age at the end of 2025. All registered patients who have not been reported as deceased, moved abroad or lost to follow-up by 31.12.2025 are included. Data collection for Pompe disease by the Swiss-Reg-NMD will start in 2026.

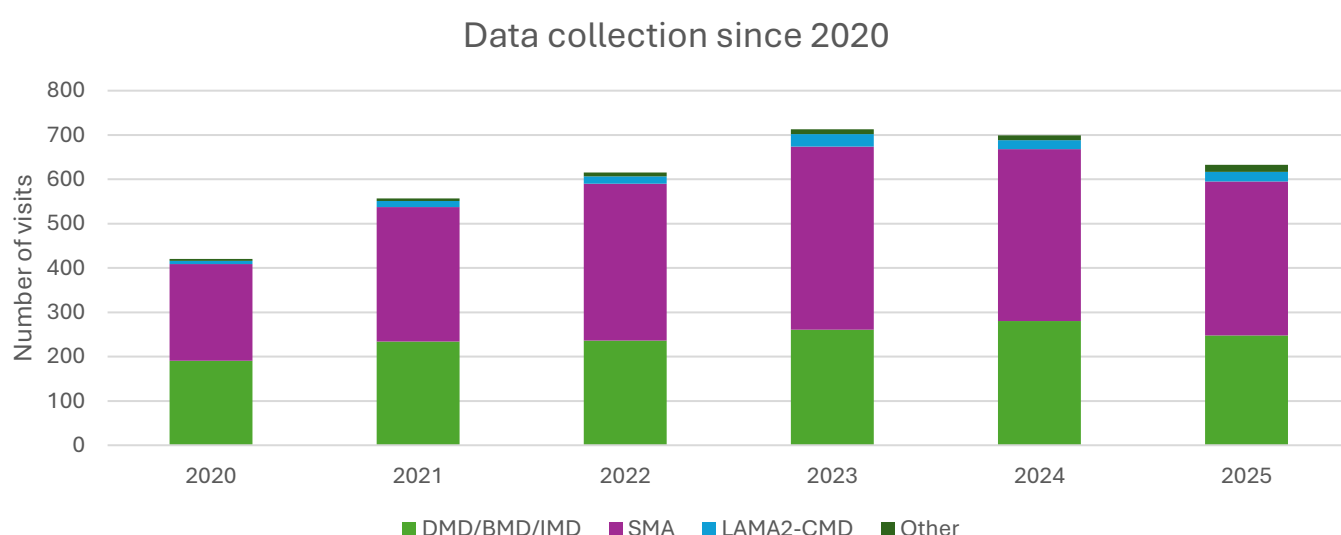


Figure 6: Development of data collection since 2020. Number of patient visits (events) reported to the registry of all patients ever registered in the registry. Data form 2025 remains to be completed. Data collection for Pompe disease by the Swiss-Reg-NMD will start in 2026. ‘Other’ includes currently Friedreich Ataxia, collagen VI-related dystrophy and limb-girdle muscular dystrophy.

Requests addressed by the Registry

Every year Swiss-Reg-NMD receives several enquiries from clinicians, researchers, pharmaceutical companies, patients, or other stakeholders. The content of these enquiries spans from individual patient data requests and epidemiological analyses to treatment evaluations and international data harmonization initiatives. Depending on the nature of the enquiries, the applicant may be required to fill in an application form entailing purpose and intended use of the data requested, as well as planned analyses, publications, measures of data security, and funding. If applicable, approval from the (local) ethics commission must be submitted together with the application form. A patient can request their own data simply via email, but a copy of the ID card or passport is mandatory. For enquiries about basic epidemiological information (e.g. total number of patients with SMA) a simplified application form is available. Every enquiry is thoroughly reviewed by the executive office regarding its purpose, methodology, and legal aspects. Depending on the nature and scope of the enquiry the steering board is involved in the approval process.

During 2025, about 30 different enquiries have been processed by the Swiss-Reg-NMD. They included requests from patients, researchers, physicians, the pharmaceutical industry, TREAT-NMD, and authorities. The reason for the data requests reached from scientific presentations, teaching activities, patient's own interest, to feasibility assessments of a research project or clinical trial. The number of requests to the Swiss-Reg-NMD doubled since 2020. This demonstrates the growth and increasing use of the registry as a research and network platform. The following figure provides insight into different types of requests reaching the registry with a specific example each from 2025.

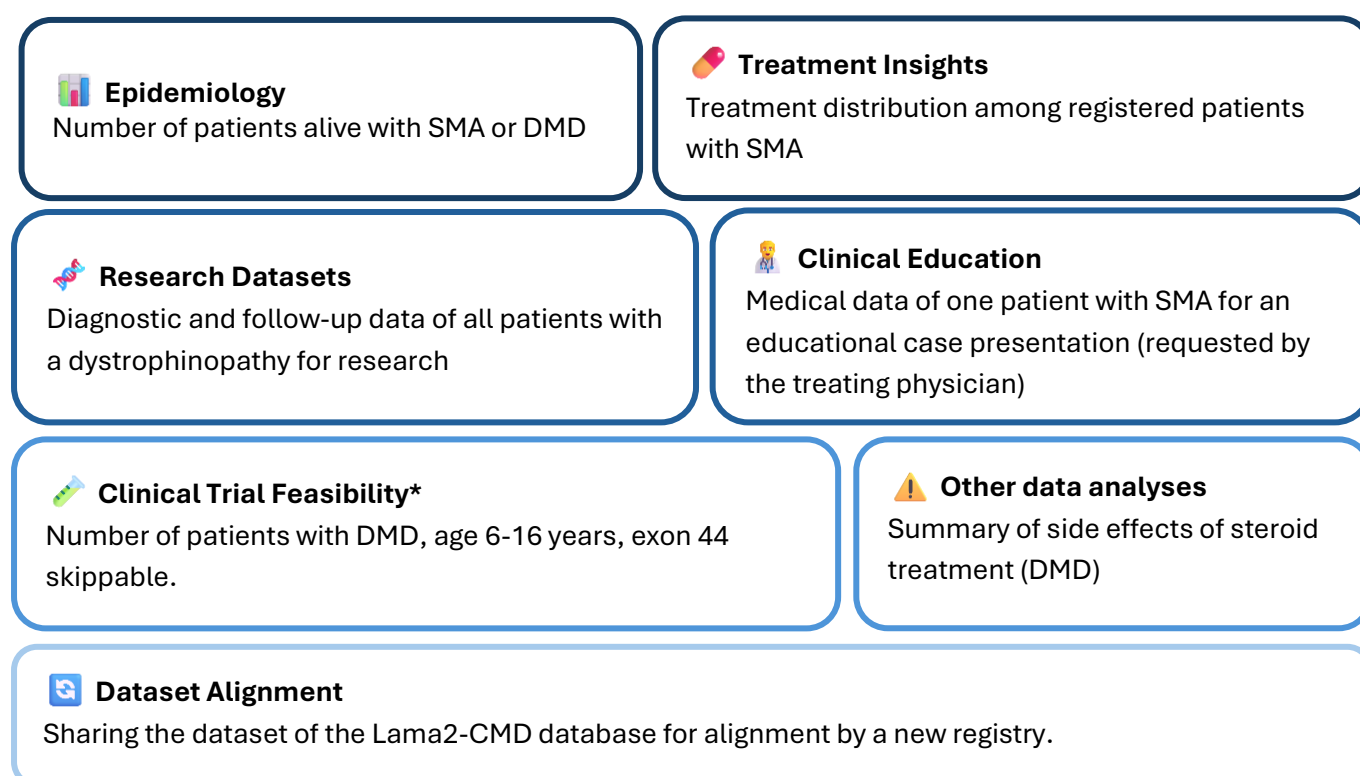


Figure 10: Categories of processed enquiries from a variety of stakeholders with examples from 2025. *The registry itself does not recruit patients for clinical trials but supports the opening of clinical trial sites in Switzerland by answering enquiries about the number of patients eligible for a particular study for feasibility analyses.

Projects and Collaborations

Infrastructure Projects

Development and setup of the database for Pompe Disease

In 2025 the executive office focused on the setup and development of the infrastructure to incorporate Pompe disease in the registry. The dataset of the former Swiss Pompe Registry led by Sanofi-Aventis Schweiz AG served as a basis for the development of a new RedCap database intended to hold the retrospective data while allowing incremental improvements for future data collection. By November 2025 a first version of the database was ready, and we requested the data of all patients who had signed the informed consent for Swiss-Reg-NMD from Sanofi-Aventis. Importing these data into the new database will be the next great step planned for the first quarter of 2026.

Guidelines

Over the past two years, the guidelines for data collection and data entry into the Swiss-Reg-NMD databases have been thoroughly revised. The new versions were sent to all data providers in July 2025. There are now three separate documents: (1) one provides information and guidelines for individuals contributing medical data to Swiss-Reg-NMD. Its purpose is to facilitate, standardize, and ensure the completeness of data collection. (2) The second document is intended specifically for individuals entering data directly into the registry's database and offers detailed instructions on data entry procedures. (3) The third document is a list of key data items (main variables) that are most frequently used in the registry's current and future analyses. This helps data providers prioritize documentation and data submission, which is of the utmost importance for the registry.

Research Projects

Disease progression and adherence to care standards in the DMD cohort in Switzerland

Lead: A. Klein, Inselspital Bern

This project funded by FSRMM⁴ aims to characterize the cohort of Swiss patients with dystrophinopathies. The expected results are fundamental to compare the effect of new treatments in a real-world setting. The project addresses two main objectives: (1) Assessing disease progression regarding ambulation, lung function, and heart function. (2) Monitoring adherence to standards of care with a focus on steroid treatment and its effects on cardiac, respiratory, and bone health. Identifying actionable hurdles and gaps in the current standard care to reduce the burden for patients and their families is part of this second objective. The project is based on registry data and a survey. In 2025, we defined the variables and data quality criteria to analyze disease progression and started the collection of missing data. The analysis of part 1 is ongoing.

Getting-ready for real-world evidence in DMD long-term follow-up 'rwDMD project'

Lead: A. Klein, Inselspital Bern

This project funded by Roche aims to implement learnings from post-marketing surveillance studies of SMA treatments to get ready for real-world evidence studies on the efficacy of upcoming DMD treatments. The proposal includes harmonization of care standards across Switzerland, evaluation of the current state, alignment of the registry data set with other European registries, improvements of data quality and data collection as well as teaching activities. By the end of 2025 we completed the evaluation and cleaning of the main variables in our database and started the comparison of our dataset with other European DMD registries.

⁴ <https://www.fsrmm.ch/>

PROMS (Pilot Project)

Lead: L. Steiner, Inselspital Bern

This project aims to introduce patient reported outcome measures (PROMs) into the Swiss standards of care. In the current pilot, a set of PROMs questionnaires are distributed to patients with SMA who are seen in the children's hospitals Bern and Zurich. The registry provides the necessary infrastructure for data collection and supports data entry.

Health Service Evaluations (on behalf of authorities / pharmaceutical companies)

Reports on Zolgensma®

During 2025, we invested significant resources in securing a follow-up agreement with Novartis for continued reporting on Zolgensma®. The main task of the Swiss-Reg-NMD according to the agreement is monitoring treatment outcome and providing an evaluation report intended for sharing with the FOPH. In exchange, Novartis supports the registry by covering part of its running costs (management & data collection) plus the labour costs associated with the reporting. The first report under this agreement is due in April 2026. It will include data pertaining to the period up to December 31, 2025. The report will show only aggregated data (identification of individual patients will not be possible).

First report on newborn screening for SMA

Lead: David Jacquier, CHUV

A report about the first year of the SMA newborn screening in Switzerland was completed and submitted to the FOPH. The report shows that 95% of positive screenings were truly positive and newborns meeting the criteria for one of the disease-modifying therapies (DMT) were treated within 21 days of birth. Early treatment is key to favorable developmental outcomes. All patients showed no or only minor symptoms by the end of the reporting period.

National and International Collaborations

Myosuisse

Myosuisse is the Swiss network of experts and organizations dedicated to the care and well-being of people living with a neuromuscular disorder and is recognized by the KOSEK. The general assembly took place at the Inselspital Bern in August 2025. Following the approval of the network's governance structure at the meeting, Swiss-Reg-NMD officially became a core member of the Myosuisse network.

Treat-NMD and Fair-DMD

The registry collaborates internationally for research projects and other initiatives. One of the main collaborators of the Swiss-Reg-NMD is TREAT-NMD⁵. TREAT-NMD is a global network of experts in the field of NMD. Its primary goal is to accelerate the development of effective treatments and establish the best in diagnosis and care for people with an NMD worldwide. Our clinical lead, Andrea Klein, is a long-standing elected member of the TREAT-NMD Global Database Oversight Committee (TGDOC) for the international registries for SMA and DMD. This committee is responsible for reviewing requests for data or recruitment support from the Global Registries Network. Our vice clinical lead, David Jacquier, is a member of the TREAT-NMD Neuromuscular Disease Advisory Committee (NMDAC), which provides quarterly updates and strategic advice to the TREAT-NMD Alliance Ltd. Board of Trustees. Their focus includes reporting on the status and advances in NMD research, clinical trials, and patient care. They also identify areas of unmet needs within the NMD field, propose task forces to address these needs, and suggest potential areas of work and projects where TREAT-NMD can add value. In 2025, Swiss-Reg-NMD participated in three projects led by TREAT-NMD:

⁵ <https://www.treat-nmd.org/>

1) A survey on behalf of a pharmaceutical company regarding data collection practices across the BMD registry network. This survey substantially increased confidence in BMD registries to provide comprehensive data on a large BMD population and to elaborate relevant research questions based on the knowledge gathered through the network. 2) TREAT-NMD conducted a survey on consent- and ethics procedures within the registries to improve data quality and be qualified by the European Medicines Agency (EMA) 3) A vote on whether the network should participate in a project mandated by the EMA to assess whether real-world data sources in DMD are fit for the purpose of regulatory decision-making. The network has approved participation in this project. A follow-up is planned for 2026.

Further, we collaborate on the Fair-DMD⁶ project. The project is about building an international Duchenne-Becker Muscular Dystrophy registry (currently Germany, Austria, Switzerland) and is led by the German Society for Pediatric Neurology (GNP). The Fair-DMD registry recently concluded the development phase and will start during 2026 into the running phase. During 2025 the Swiss-Reg-NMD was involved in two working groups: 1) dataset definition and 2) governance structure and data sharing policy development.

Swiss Rare Disease Registry (SRDR) & SwissPedNet

The Swiss-Reg-NMD collaborates with the Swiss Rare Disease Registry (SRDR)⁷. Both registries are hosted at the ISPM at the University of Bern. Their employees work closely together, which allows sharing resources and knowledge on various topics.

We also collaborate with SwissPedNet, the Swiss research network of clinical pediatric hubs, which facilitates and supports pediatric clinical research across Switzerland. As part of SwissPedNet, SwissPedRegistry, based at the ISPM in Bern, provides expertise in the development and management of pediatric registries and cohorts. This network and expertise provide valuable support for Swiss-Reg-NMD and facilitate collaboration with pediatric hospitals and registries across Switzerland.

Miscellaneous

Rare disease legislation

During the last year a legislation draft aiming to regulate the measures to tackle rare diseases in Switzerland has been developed and published by FOPH (*Bundesgesetz über Massnahmen zur Bekämpfung seltener Krankheiten*⁸). Key features of the legislation are establishing a rare disease registry, providing financial support for specialised care delivery structures as well as information and awareness activities. The Swiss-Reg-NMD submitted a statement during the consultation procedure from September 12 to December 12 (2025).

⁶ Fair-DMD: <https://clinicaltrials.gov/study/NCT07402122>

⁷ <https://www.raredisease.ch/>

⁸ https://www.fedlex.admin.ch/filestore/fedlex.data.admin.ch/eli/dl/proj/2024/78/cons_1/doc_1/de/pdf-a/fedlex-data-admin-ch-eli-dl-proj-2024-78-cons_1-doc_1-de-pdf-a.pdf

Dissemination & Networking

Publications and articles

Real-world data on the effect of long-term treatment with nusinersen over > 4 years in a cohort of Swiss patients with spinal muscular atrophy. Tscherter A, Steiner L, Broser PJ, Enzmann C, Galiart E, Henzi BC, Jacquier D, Mathis AM, Neuwirth C, Ramelli GP, Ripellino P, Scheidegger O, Schreiner B, Schwarz EI, Stettner GM, Klein A; Swiss-Reg-NMD Group. Clin Neurol Neurosurg. 2025 Aug;255:108983. doi: 10.1016/j.clineuro.2025.108983. Epub 2025 May 26. [PMID: 40451126](#).

Education and participation in children and adolescents with Duchenne muscular dystrophy in Switzerland. Henzi BC, Baumann D, Michalopoulou E, Erni SJ, Steiner L, Lötscher N, Tscherter A, Klein A; Swiss-Reg-NMD Group. Eur J Paediatr Neurol. 2025 May;56:107-114. doi: 10.1016/j.ejpn.2025.03.010. Epub 2025 Mar 22. [PMID: 40349652](#).

Swiss-Reg-NMD: Le registre suisse pour la maladie neuromusculaires. Mathis AM, ASRIMM patient magazin 'entre-nous', printemps 2025, [Website ASRIMM](#)

Workshops

Establishing expert care recommendations for LAMA2-RD: A prototype for the development of congenital muscular dystrophy subtype-specific care guidelines. 283rd ENMC International Workshop, Hoofddorp, The Netherlands, January 17th – 19th 2025, A.A. Zambon, A. Klein, A. Sarkozy, A.R. Foley,

Presentations and posters

Molecular Therapies: Opportunities and Challenges in the Treatment of Patients with Neuromuscular Diseases, A. Klein, Kongress *pädiatrie schweiz* (SGP), Bern, May 2025

Efficacy of Nusinersen treatment over >4 years in Swiss patients with Spinal Muscular Atrophy: A multicenter observational study. L. Steiner, Kongress *pädiatrie schweiz* (SGP), Bern, May 2025

Advancing the Understanding of Dystrophinopathies in Switzerland: Disease Progression & Standards of Care. N. Pfenninger, Kongress *pädiatrie schweiz* (SGP), Bern, May 2025

LAMA-2 und Neuropsychologie: eine Fallvorstellung. B. Kohler, Jahrestagung vom Verein Kinder- und Jugendneuropsychologie, May 2025

Medizin: Innovationen SMA in der Schweiz. G. Stettner, SMA Day, Nottwil, Jun 2025

Update on Swiss-Reg-NMD. A. Mathis, General Assembly Myosuisse, Bern, Aug 2025

Das Schweizer Register für neuromuskuläre Erkrankungen (Swiss-Reg-NMD); Mehrwert für Patient*innen, Familien, Fachpersonen und Forschung. D. Baumann, Duchenne Konferenz, Nottwil, Sept 2025

Newborn screening for spinal muscular atrophy: First anniversary in Switzerland on March 1st 2025. D. Jacquier, Congress of the World Muscle Society (WMS), Vienna, Oct 2025

Symptomatische und kausale Therapien der Duchenne Muskeldystrophie im Wandel der Zeit. G. Stettner, Neuromuskuläres Symposium KISPI Zürich, Nov 25

Swiss cohort of adult patients receiving nusinersen at timepoint of data extraction: March 2025. O. Scheidegger, Congress of the Swiss Federation of Clinical Neuro-Societies (SFCNS), Lausanne, Dec 2025

Networking

- Swiss-Reg-NMD information desk in exhibition hall at the SMA Day, Nottwil, June 25
- Myosuisse General Assembly, Inselspital Bern, Bern, Aug 25
- Nacht der Forschung, exhibition room about: Medizinische Register, Universität Bern, Bern, Sept 2025
- Jahreskongress Gesellschaft für Neuropädiatrie (GNP), Kursaal, Bern, Oct 2025

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