EURO-NMD
Registry Hub
European Reference Network
for neuromuscular diseases

DATA ACCESS POLICY

This Data Access Policy (DAP) document applies to the European Reference Network (ERN) Registry for Rare Neuromuscular Diseases (EURO-NMD Registry). It covers the composition of the Data Access Committee (DAC) as well as the entire process to be followed for requesting access to the data captured in the EURO-NMD registry.

Background

The EURO-NMD registry is the European registry for all patients (paediatric and adults) with rare neuromuscular diseases (NMDs). The registry is affiliated to the European Reference Network for rare neuromuscular diseases (ERN EURO-NMD).

The aim of the registry is to improve quality of care, including the diagnosis, treatment and prognosis of patients with rare neuromuscular disorders and to enable research by gathering data from a large number of NMD patients and providing regulated data access following this Data Access Policy.

The EURO-NMD registry consists of a collection of common data elements for all patients followed at the participating Healthcare Providers (HCPs) of the ERN EURO-NMD combined with disease-specific data elements for all thematic disease groups covered by the ERN EURO-NMD. It incorporates the set of common data elements for rare disease registration released by the European Platform on Rare Disease Registration (EU RD Platform CDE), key performance indicators (KPIs) of care processes and patient-reported outcome measures (PROMs).

The EURO-NMD registry complies with the legal, ethical and privacy standards required within Europe. Patient data is protected in full compliance with the European Union (EU) regulations on the processing and free movement of personal data as well as with all pertinent national laws and regulations of the Member States. Written informed consent for participation in the registry is obtained from all patients and personal identifiers are removed and replaced by a pseudonym prior to data being submitted to the Registry.

The registry database and data are hosted in the European Union. The Registry is hosted at: Clinical Trials Unit, Faculty of Medicine and Medical Centre, University of Freiburg, Freiburg, Germany. The centres participating in the EURO-NMD Registry have concluded an agreement on joint protection responsibility according to art. 26 of the General Data Protection Regulation (GDPR).

For more information see: https://registry.ern-euro-nmd.eu/.

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Tasks of the Data Access Committee

The overall aim of the Data Access Committee (DAC) is to promote the research use of the data that are being collected in the EURO-NMD registry through a transparent and simple approach ensuring the long-term sustainability of the project. The DAC should advise on the maintenance of the highest levels of custodianship of the data. Whilst it should have a good knowledge of ethics and data protection, it should not act as another 'research ethics committee' which is a responsibility that rests at the level of the data controllers. The DAC should:

- Check that the proposed work complies with the terms and conditions of the ethics approval provided to the EURO-NMD registry.
- Look for evidence that the third-party requesting data is appropriately qualified for use of the data.
- Advise on improving the projects and any overlaps with ongoing projects.
- Ensure that the effort of all those involved is appropriately acknowledged.
- Aim to respond to all data requests promptly.
- Communicate to the requestor with appropriate feedback.
- Be aware of their own conflicts of interest.
- Treat all data requests confidentially.

Composition of the Data Access Committee

The current composition of the Data Access Committee (DAC) consists of the Steering Committee of the EURO-NMD Registry project and the Chairs from each of the 5 thematic disease groups covered by the ERN EURO-NMD on an ad-hoc basis.

The EURO-NMD Registry project Steering Committee consists of the following members:

Neurologist and Coordinator of the ERN EURO-NMD (Chair): Teresinha Evangelista, Pitie Salpetriere University Hospital (AP-HP), Paris.
Neurologist and Scientific Coordinator of DM-Scope Myotonic Dystrophy Registry: Guillaume Bassez, Pitie Salpetriere University Hospital (AP-HP), Paris.
Neurologist and Clinical Advisor of the ERN EURO-NMD: Antonio Atalaia, Pitie Salpetriere University Hospital (AP-HP), Paris.

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Neurologist and Chair of the ERN EURO-NMD Research Advisory Board: Hanns Lochmüller, University Medical Centre Freiburg (UKLFR).
Bioinformatics expert: Peter-Bram t'Hoen, Radboudumc University Medical Center Nijmegen
IT specialist: Adrian Tassoni, University Medical Centre Freiburg (UKLFR)
FAIR data expert: Nawel Lalout, Radboud University Medical Center (Radboudumc)
Patient representative and ERN EURO-NMD ePAG: Dimitrios Athanasiou, World Duchenne Organization (WDO)
Patient representative and Chair of the World Duchenne Organization: Elizabeth Vroom, World Duchenne Organization (WDO)
Patient representative and Chair of the ERN EURO-NMD Patient Advisory Board: Francois Lamy, French Muscular Dystrophy Association (AFM-Téléthon)
Patient Representative, AI expert and Chair of the Duchenne Data Foundation: George Paliouras (DDF)
Project Manager: Paraskevi Sakellariou (DDF)
Data manager: Dagmar Wandrei, University Medical Centre Freiburg (UKLFR)
Project and Communication manager: Suzie Ann Bakker, Duchenne Data Foundation (DDF)
Senior Project Manager of the ERN EURO-NMD: Carla D'Angelo, Institute of Myology, Paris (AIM).

Registry authorized users

The EURO-NMD registry is a web-based platform subject to role-based authorization privileges and controls. Access of authorized users to the registry is controlled by assignment of a secure, individualized password. Authorised users are issued with a unique username and password enabling them the appropriate level of access to the system

The following broad groups of stakeholders will require access to the EURO-NMD registry platform:

Registry Coordinator & Management Team	Has full access to all data and provides access to authorized users
Hospital Coordinators (principal investigators)	Has full access to all cases at their centre and approves or denies access requests from centre

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	users.
ERN Workgroup leads	Has full access to all cases of the disease group covered by the relevant workgroup.
Clinical Contributor	Has full access to all the cases of their center and approves or denies access requests from local team members.
Patient	Has access to their own individual data. Can complete on-line questionnaires, gain information and understanding of what has been done with their data (who had/has access to their data and for which purpose).

Stakeholders entitled to Request Registry Data

The following stakeholders are entitled to request data from the EURO-NMD registry in order to contribute to projects whose objectives are directly connected to improve healthcare provision to individuals living with rare neuromuscular diseases:

- Researchers contributing to the EURO-NMD registry
- Patients contributing own data to the EURO-NMD registry
- Non-contributing researchers
- Pharmaceutical companies
- Health authorities
- Policy, supervisory or regulatory agencies
- Payers/insurers
- Non-Governmental Organisations
- Patient organizations

Note: Private companies data access should be submitted to the negotiation and execution of a separate agreement.

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Categories of requestable data

The stakeholders entitled to request registry data can request the following type of data:

Aggregated data	All data derived from the Registry Data, no longer attributable to any individual Patient. This can include descriptive statistics, comparative statistics, graphs, presentations, counts or any other derived or aggregated data set;
Individual-level data	All data derived from the Registry Data that are attributable to an individual Patient.

The data access authorisation levels can be found in detail in Annex A. All data access requires authentication.

All data in the registry are pseudonymized, in the definition of GDPR; the registry does not contain directly identifiable data elements. The pseudonymization key lies with clinical contributors. Stakeholders that are given access to the data should not make any attempt to identify a participant in the registry.

Ownership of the data

In the EURO-NMD registry, the patient participant (who is the 'data subject') is the primary owner of the data and must give explicit informed consent prior to participation in the Registry and to the use of such data for research and other purposes. In case the patient participant is under the age of majority, or if the data subject is physically or legally incapable of giving consent, parent(s) or legal guardian(s) is/are the primary owner(s) of the data and are authorised to provide consent for the data collection and all processing activities.

The institution of the clinician participant who has entered the data is the owner of the data of that patient participant.

When processed, the data become research data and are then the intellectual property of the investigator.

The healthcare provider hosting the Coordinating Centre of the ERN EURO-NMD is the owner of the EURO-NMD registry platform.

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Research and data analysis

The Registry is funded within the 3rd Health Programme of the European Union and receives support from the World Duchenne Organisation (WDO), Duchenne Data Foundation (DDF), Institute of Myology (AIM), and the French Muscular Dystrophy Association (AFM-Téléthon).

The data in the EURO-NMD registry shall undergo analysis at regular intervals by the Project Management Team for detailed data consistency evaluations. This analysis will focus on overall data accrual, content, quality, and headline descriptions of care. This analysis will not require approval from the DAC but shall be performed closely with oversight of the DAC to provide progress reports.

All other analysis will require completion of a Data Access Request Form to be reviewed by the Data Access Committee where appropriate and the signing of a Data Transfer Agreement regulating the terms and conditions for the use of data, including:

- Commitment to ensure that the data will be used for the purpose intended and approved
- Commitment to respect the EU legislation
- Commitment to no attempt to re-identification
- Commitment to no attempt to directly contact the patients.

Prior to any application being prepared/submitted, investigators should first review the data elements and data dictionary posted on the Registry website to verify that critical data are available for their proposed study. In addition, proposals must not have any major overlap with other approved research proposal. A list of previously approved research proposals, or studies that are in progress, are available on the Registry website.

Process for seeking access to the data

- Applicants shall need to complete the online Data Access Request Form for every proposed research question/project.
- The EURO-NMD registry Management Team will undertake an initial review within 3 weeks of submission and forward to the DAC; if the application has not been satisfactorily completed, the management team will request clarification or further information.

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- The DAC meets on a monthly basis. Applications which are complete and have addressed all applicable clarifications will be discussed at the next available DAC meeting.
- The DAC shall provide their feedback using the Feedback Form within one week of the meeting. The DAC may ask the requesting investigator for clarifications related to their proposals before a final decision.
- Following approval, applicants shall need to sign and comply with the Data Transfer Agreement.
- If a fee is requested to access the data, the management team will notify the applicant prior to release of prepared data.
- Some requests will not require DAC review but must still be made in writing, on the appropriate form, stating the need and purpose of the request.
- In case the contents of a new application overlap with an existing active application, the investigators of the two applications will be jointly advised to discuss the overlap.

The Registry only provides access to data for which:

- documented and valid informed consents have been obtained from patients (or their legal representatives); when an industry representative applies for data, only data from patients who consented to commercial use will be granted; and
- required documentation regarding requests for Access to Registry Data is received, on the appropriate forms, including but not limited to the data access request form; and
- DAC approval has been obtained to allow investigator(s) to access and use the data for a specific study proposal (if required); and/or
- Ethics' committee or institutional review board authorization has been obtained as required by Investigator prior to initiating the research as required.

Note: Requests seeking to use the Registry to recruit participants for a study or for additional data collection should be addressed to the Steering Committee.

All documents are accessible on the EURO-NMD registry website (https://registry.ern-euro-nmd.eu/) and through the EURO-NMD registry project management team.

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Governance review

This document will be reviewed every two years from the implementation but may be reviewed and updated more frequently as required, e.g. in the event of operational changes or following significant changes to any applicable laws.

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Annex A: Stakeholders entitled to request data & data access authorization levels.

Stakeholder	DAC permission required	Cohorts	Data elements	Data access level	Authentication	Fees
Contributing researcher	No	Own patients	All	Individual-level	Yes	No
	No	All patients	All	Aggregated	Yes	No
	Yes	All patients	All	Individual-level	Yes	No
Contributing patient	No	Own data	All	Individual-level	Yes	No
	No	All patients	All	Aggregated	Yes	No
	Yes	All patients	All	Individual-level	Yes	No
Non-contributing researcher	No	All patients	All	Aggregated	Yes	No
	Yes	All patients	All	Individual-level	Yes	No
Industry	No	All patients consenting to commercial use	CDE	Aggregated	Yes	Yes

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	Yes	**All patients consenting to commercial use**	**CDE + selected disease history/ intervention/ outcome data**	**Individual-level**	**Yes**	**Yes**
Health Authorities	**No**	**All patients**	**CDE**	**Aggregated**	**Yes**	**No**
	Yes	**All patients**	**CDE + selected disease history / intervention/ outcome data**	**Aggregated**	**Yes**	**No**
Policy, supervisory or regulatory agencies	**No**	**All patients**	**CDE**	**Aggregated**	**Yes**	**No**
	Yes	**All patients**	**CDE + selected disease history / intervention/ outcome data**	**Individual-level**	**Yes**	**No**
Payers/insurers	**Yes**	**All patients**	**CDE**	**Aggregated**	**Yes**	**Yes**
Non-Governmental Organisations	**Yes**	**All patients**	**CDE**	**Aggregated**	**Yes**	**Yes**
Patient Organisations	**No**	**All patients**	**All**	**Aggregated**	**Yes**	**No**

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	Yes	**All patients**	**All**	**Individual-level**	**Yes**	**No**

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