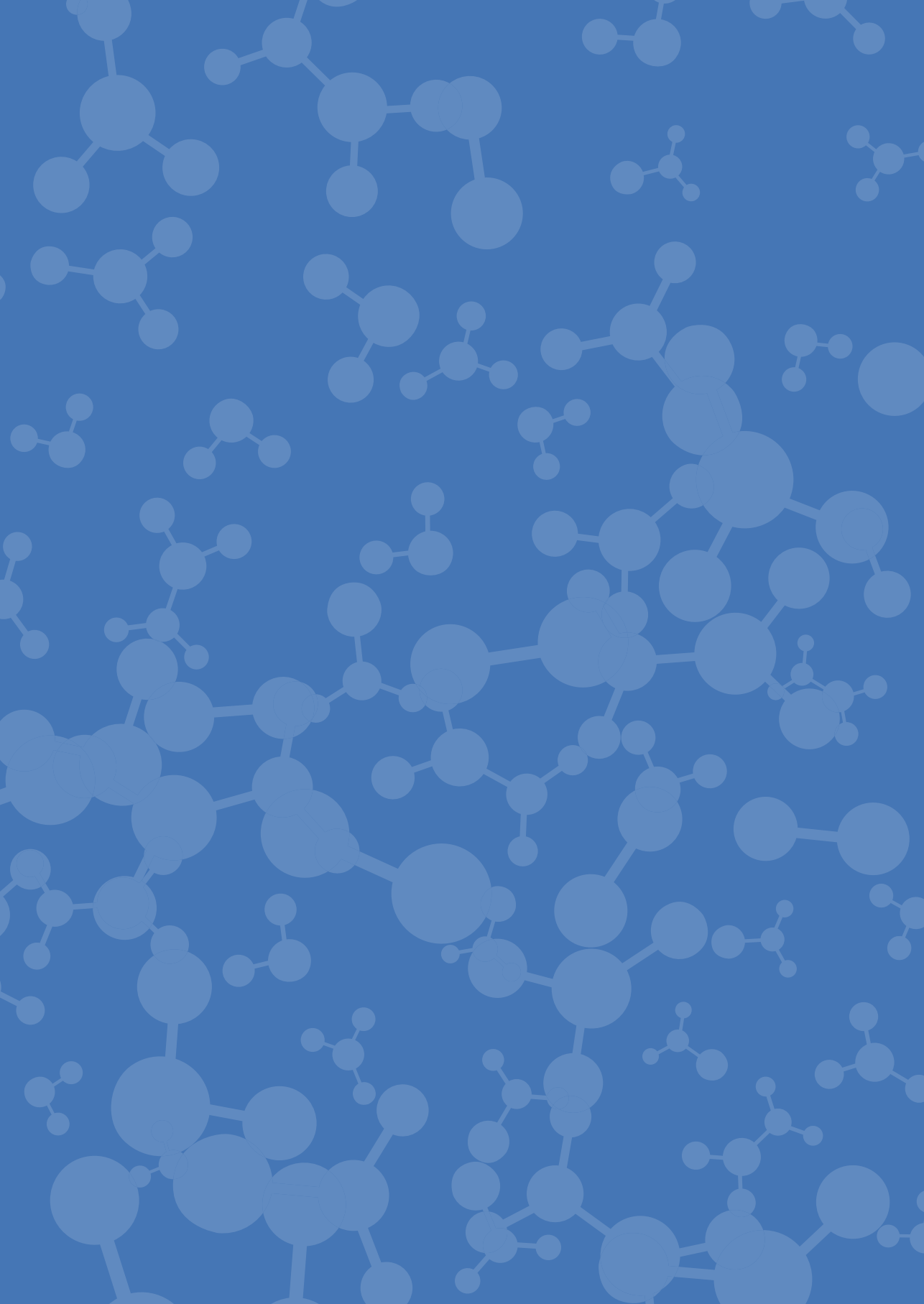
ENMC Impact Report 2025

Our year in highlights

EUROPEAN NEURO MUSCULAR CENTRE



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Our year in highlights

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CENTRE

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1 Message from Dr Ingeborg Meijer, Chair of the Executive Committee

In 2025, connecting people was at the very heart of everything the European Neuromuscular Centre (ENMC) did. More than a theme, it was a lived experience — one that shaped our meetings, our collaborations, and our vision for the future of neuromuscular research and care.
This spirit of connection was strongly felt during the ENMC General Assembly Meeting, where shared decision making took centre stage. The discussions highlighted, once again, how essential the role of patients is in shaping research priorities, clinical practice, and policy. Bringing together patient representatives, researchers, clinicians, and other stakeholders in an open and respectful dialogue strengthened mutual understanding and reinforced our shared commitment to truly patient-centred care.

Throughout the year, ENMC continued to connect its broad and diverse network: patient organisations, scientists, clinicians, and members of our Company Forum. These connections are the foundation on which progress in neuromuscular diseases is built. Our workshops remain a prime example of this philosophy in action — providing a safe, inspiring, and collaborative environment where ideas can be exchanged freely and where different perspectives enrich scientific debate and consensus building.

Connection was also tangible at the World Muscle Society (WMS) Congress in Prague. The ENMC booth was lively and well attended, serving as a natural meeting point for old friends and new faces alike. It was heartening to see how the ENMC office team increasingly encountered familiar colleagues across the global neuromuscular community — clear evidence of lasting relationships built over time.

Connectedness also means ensuring that the next generation finds its way to ENMC. In 2025, we received a record number of applications for the ENMC Mid-Career Mentoring Programme, reflecting the growing engagement of younger researchers and clinicians. Supporting these future leaders in neuromuscular diseases is one of ENMC's most important investments in the years to come.

For me personally, 2025 marks a moment of transition. After ten years as a member of the Executive Committee, this is my final activity for ENMC. I now pass the chair's gavel to Kate Adcock with great confidence. I am deeply grateful for the connections made over the past decade and wish ENMC a strong, connected, and inspiring future.

Dr Ingeborg Meijer,
Chair of the Executive Committee,
Representative of Spierziekten Nederland,
The Netherlands

2 The mission of the ENMC

Over 33 years ago, a group of scientists and clinicians, together with parents of children affected by neuromuscular conditions, launched the European Neuromuscular Centre (ENMC). They had in mind the ultimate goal to improve diagnosis, accelerate the search for effective treatments and improve the quality of life of people with neuromuscular conditions. To achieve this goal, it was, and still is, of utmost importance that experts in this field of (ultra) rare disorders share their knowledge and experience and collaborate in research worldwide.

The ENMC encourages and facilitates this collaborative aim through the organisation of small interactive workshops for multidisciplinary groups of researchers, clinicians and persons affected by neuromuscular conditions, a unique concept in the scientific community.

ENMC Mission Statement

The mission of ENMC is to encourage and facilitate communication and collaboration in the field of neuromuscular research with the aim of improving diagnosis and prognosis, finding effective treatments and optimising standards of care to improve the quality of life of people affected by neuromuscular disorders.

"Connecting people"

3 2nd ENMC General Assembly Meeting

On 4-5 April 2025, stakeholders from across the ENMC gathered in Hoofddorp, the Netherlands, for the second General Assembly Meeting (GAM). Attended by the Executive Committee, Research Committee, Associated Partners, and members of the Company Forum, this event marked an important moment in fostering collaboration and innovation within our neuromuscular community.

During the GAM, we explored the topic of shared decision-making in clinical trial design. The session began with a presentation by Ingeborg Meijer, Chair of the ENMC, who kicked off a discussion on clinical trial design that built upon insights from the 235th ENMC workshop and the White Paper published in 2020.

Ingeborg Meijer, chair ENMC, explained the session on shared decision-making in clinical trial design.

The round table discussion centered around the following model:

Clinical Trial Design		
1 Patient participation in trial design	2 Broadening trial participation	3 Impact of trial design on participants quality of life
Who should be involved? How can they be involved? At what stages should they be involved? Challenges/Opportunities?	How can trials incorporate transition from one centre to another? How can sponsors broaden trial participation and make it more inclusive? Challenges/Opportunities?	How can trial design reduce the impact of participation in daily living? Can new technologies be used/developed? Challenges/Opportunities?

Elizabeth Vroom (World Duchenne Organisation) is presenting the findings from her group's discussion.

General summary

The discussions on patient participation were dynamic and insightful, reflecting the diverse expertise of stakeholders.

Topic 1 - Patient participation in trial design: The overall consensus is that patients should be involved from the outset and throughout all stages of the process. Participation should include a diverse group of patients from various types of organisations. However, organising such contributions in a systematic way is challenging. It requires careful planning, training, and time, while also managing expectations. When well-organised, patient involvement can enhance public trust and acceptance of both the initiation and outcomes of clinical trials.

A group discussion involving different stakeholders.

Topic 2 - Broadening trial participation: Broadening participation in clinical trials could help raise awareness and improve inclusion of underserved populations. This would require broad eligibility criteria to ensure real-world relevance of results, including considerations for health technology assessment (HTA) and quality of life from the outset. Therefore, comprehensive data collection and the use of validated endpoints should be implemented from the beginning. Such an approach would benefit from a network of trial centres operating within a hub-and-spoke model, ensuring no opportunities are missed and enabling remote assessments where appropriate. Transitioning patients between centres may lead to some loss in the consistency of outcome assessments; however, losing a patient entirely represents a far greater loss. This issue is particularly relevant during the transition from pediatric to adult care.

All participants of the 2nd ENMC General Assembly Meeting.

Topic 3 - Impact of trial design on participants' quality of life

Clinical trial sites need to be set up to run modern, patient-friendly studies. That means giving staff the space and tools they need, using remote/virtual ways to check in with participants (including patient reported outcome measures - PROMs), and adopting wearables or other innovative tech but only when those tools produce reliable, validated outcomes and do not add burden for patients. After the trial, participants should be asked how the trial affected their quality of life so future improvements can be made. Realistically, red tape and approvals for new tools are still hurdles.

Conclusion

The roundtable discussions — marked by lively, in-depth, and diverse contributions — highlighted both the importance and the complexity of integrating shared decision-making into clinical trial design. As with any complex issue, there are no simple solutions or one-size-fits-all answers. Given the strong interest and valuable insights shared, it was agreed that this theme had significant potential to be developed into a full ENMC workshop to explore some of the key aspects raised. The ENMC invites interested participants to submit workshop applications aligned to this theme.

The positive feedback we received underscores the success of the GAM. Building on this momentum, the ENMC is pleased to announce that we're already planning for the 3rd General Assembly Meeting in 2027, further solidifying our commitment to collaboration and advancement within the field of NMD research and treatment.

4 The ENMC workshops in 2025

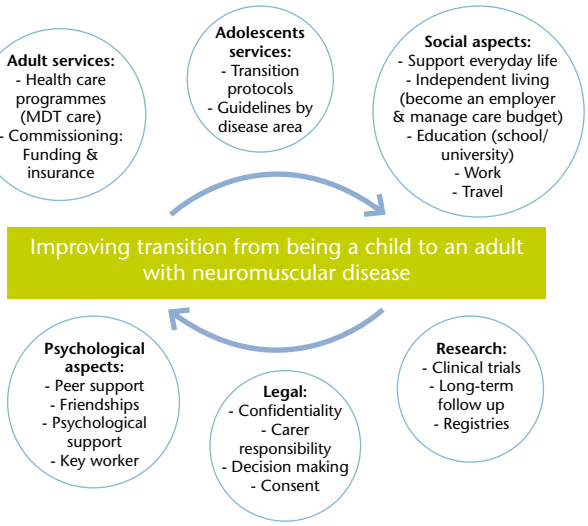
The year 2025 saw a remarkably high number of workshop applications (19) submitted to the ENMC, including four re-applications. Nine of these were selected for support and are scheduled to take place in 2026.

4.1 Themed Workshops

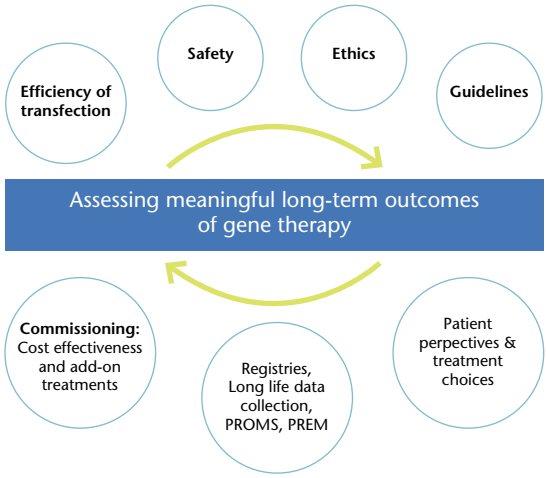
In addition to the regular open workshop applications, where the topics are proposed by the workshop organisers, the ENMC also offers "Themed Workshops". For this type of workshop, the ENMC's stakeholders have identified two specific themes that are prioritised based on the needs of neuromuscular patients and related research.

At the 2nd ENMC General Assembly Meeting in April 2025, the Executive Committee, Research Committee, Associated Partners, and members of the Company Forum selected two themes:

1 Improving transition from being a child to an adult with neuromuscular disease



2 Assessing meaningful long-term outcomes of gene therapy



The themes are broad; therefore, the ENMC does not expect all sub-categories to be covered in a single workshop application. Organisers who wish to address one of these themes and apply for an ENMC workshop may focus on one or more topics related to the theme. Organisers should clearly define the rationale, background, objectives, and expected deliverables of the workshop, in accordance with the ENMC Workshop Application Guidelines and the additional Guidelines for Themed Workshop Applications. The next submission deadline for themed workshop applications is: **1 March 2027.**

4.2 Summary of ENMC workshops held in 2025

Workshop no./date and format	Topic	Workshop leaders
Workshop no. 283 17-19 January 2025	Establishing expert care recommendations for LAMA2-RDs: a prototype for the development of congenital muscular dystrophy subtype-specific care guidelines.	Dr Reghan Foley, Prof. Andrea Klein, Dr Anna Sarkozy, Dr Alberto Zambon
Workshop no. 284 24-26 January 2025	Cognitive and behavioral abnormalities in pediatric DM1 – what should we measure in preparation for clinical trials?	Prof. Valeria Sansone, Prof. Nicholas Johnson
Workshop no. 285 31 January-2 February 2025	SMN-related neurodevelopmental disorder: type 1 Spinal Muscular Atrophy and the brain.	Prof. Susana Quijano-Roy, Prof. Giovanni Baranello, Dr David Gómez Andrés, Prof. Michelle Farrar
Workshop no. 286 7-9 March 2025	Muscle imaging: artificial intelligence, automatic segmentation and imaging data sharing in neuromuscular disease (THEMED WORKSHOP).	Prof. Volker Straub, Dr Hermien Kan, Dr Jodi Warman Chardon, Prof. John Vissing
Workshop no. 287 28-30 March 2025	Harmonisation and federated analysis of Myotonic Dystrophy registries to model heterogeneous disease trajectories.	Prof. Peter-Bram 't Hoen, Prof. Karin Faber, Prof. Nicolas Johnson, Dr Guillaume Bassez
Workshop no. 288 16-18 May 2025	Towards better diagnosing, understanding and treating gastrointestinal symptoms in myotonic dystrophy.	Dr Hilde Braakman, Prof. Luca Pastorelli, Prof. Giovanni Meola, Prof. Benedikt Schoser
Workshop no. 289 26-28 September 2025	Assessing and managing emerging AAV related toxicities after gene therapy for neuromuscular disorders.	Prof. Francesco Muntoni, Prof. Carsten Bönnemann, Prof. Hildegard Büning
Workshop no. 290 21-23 November 2025	Developing Standards of Care for patients with Becker Muscular Dystrophy (BMD).	Prof. Michela Guglieri, Prof. Elena Pegoraro, Dr Erik Niks, Prof. Ros Quinlivan

Note: The workshop number (no.) is given once an application is officially approved and does not always reflect the order in which workshops are held.

283rd ENMC International workshop: Establishing expert care recommendations for LAMA2-RDs: a prototype for the development of congenital muscular dystrophy subtype-specific care guidelines

Background

LAMA2-related dystrophies (LAMA2-RDs) are one of the most common forms of congenital muscular dystrophy and are caused by mutations in the LAMA2 gene. The LAMA2-RDs are most commonly characterised by early onset muscle weakness but can present with different clinical severity, ranging in age at presentations of first symptoms from birth to adulthood and can be associated with symptoms and manifestations of disease in addition to muscle weakness, including respiratory and nutritional difficulties, tightness of the joints (so-called 'contractures') and curvature of the spine. While currently there is no cure, symptom management is key, and several promising therapies are under development. Because of the rarity and complexity of the LAMA2-RDs, there is urgent need for LAMA2-RD-specific clinical care guidelines, to improve care and support clinical trial readiness.

Aims of the Workshop

Aim 1: Gather international experts to review and summarise current evidence in the field of diagnosis, management and care of LAMA2-RDs to identify knowledge gaps.
Aim 2: Suggest expert care recommendations and reach an agreement on the best methods to develop care guidelines for LAMA2-RD.
Aim 3: Create a model which can work as a prototype for the development of care guidelines for further types of congenital muscular dystrophies (CMDs).

Workshop Deliverables

The workshop participants reviewed and agreed on several expert care recommendations and suggestions for patients with LAMA2-RDs. Clear strategies how to share this information among researchers, doctors and patient communities were also discussed and decided upon.
The participants also felt that the care guidelines should be practical and readily available and up to date with the suggestion of creating a 'live' document (an open-access, online document that can be frequently updated).

Impact for the patients and their families

Identifying expert care recommendations for LAMA2-RDs would help patients to receive more standardised and optimised care. Patients/families could benefit from improved care plans, better support, as well as clearer and more uniform information about available treatments and management. Standardised care would also help to design and deliver future clinical trials.

Participants of the 283rd ENMC International workshop on LAMA2-RD.

284th ENMC International workshop: Cognitive and behavioural abnormalities in paediatric DM1 – what should we measure in preparation for clinical trials?

Background

Myotonic Dystrophy type 1 (DM1) is one of the most variable neuromuscular disorders with onset ranging from the neonatal period to late adulthood. In all cases it is a disease that causes symptoms throughout the body, including the brain. Cognitive-behavioural aspects of adult-onset DM1 have been extensively described. For paediatric onset DM1 however, the trajectory of cognitive-behavioural development and the relation to brain involvement has not yet been characterised in depth.

The patient's voice was well covered at this important meeting by Marco Codegoni, Jorg van Gent, Bas Haasakker, Davide Sala and Christina Motton.

Aims of the Workshop

Aim 1: To define a common set of diagnostic tests to address cognitive and behavioural issues in children living with congenital and childhood DM1 that are age appropriate.
Aim 2: To provide a minimal core testing protocol to be administered at baseline and over time to improve the care of patients.
Aim 3: To discuss potential biomarkers of brain involvement that could be used in clinical trials based on current evidence and expert consensus.

Workshop Deliverables

Consensus was reached on disease-onset classification: Congenital-onset DM1 is defined as disease onset < 1 month of age and childhood-onset is defined as disease onset after 1 month from birth until 18 years of age. Altogether, all children aged < 18 should be referred to as having paediatric DM1. Literature reviews that were presented showed a consistent profile of cognitive-behavioural problems, that were also clinically recognised. General intelligence, visuo-spatial functions, attention and executive functions were mostly affected.
It is important to harmonise cognitive-behavioural assessment for: (i) screening of at-risk children who should be directed to genetic counselling, (ii) optimising international care and clinical management regarding cognitive-behavioural wellbeing during different developmental stages (iii) designing reliable, feasible and sensitive outcome measures in anticipation on clinical trials. Even though harmonising cognitive testing protocols proved to be challenging, a consensus was reached on a core cognitive testing battery to be administered internationally. It was also recognised that a combination of problems with attention, behaviour, and executive function should be considered as potential symptoms of childhood onset DM1 in the absence of muscle weakness.

Impact for the patients and their families

Harmonising cognitive testing protocols will optimise care and clinical management internationally and will allow for multicentre natural history studies which will provide a more in-depth insight of brain involvement. Patients' perspectives were taken into account when developing the testing protocol.

285th ENMC International workshop: SMN-related neurodevelopmental disorder: type 1 spinal muscular atrophy and the brain

Background

Due to significant improvements in medical care and remarkable therapeutic innovations in recent years, survival and motor outcomes in SMA have drastically changed. This is especially apparent in those who inherit the most severe SMA genotypes, for whom the new treatments may be lifesaving or stabilise their motor, breathing and/or feeding outcomes. However, it has been noticed that a proportion of treated children with favourable motor response show impaired cognitive or social skills in the first years of life, or have a delay in their communication abilities compared to their peers, or behavioural particularities. Currently, it is not fully understood how common these differences are, which areas of development are most affected, what causes these differences, or whether new SMA treatments may impact their occurrence.

Organisers of the 285th ENMC International workshop on SMA and the brain. From left to right: Dr David Gómez Andrés (Spain), Prof. Susana Quijano-Roy (France), Prof. Michelle Farrar (Australia), Prof. Giovanni Baranello (UK).

Aims of the workshop

Aim 1: Bringing experts together: clinicians, scientists, researchers, and advocacy group representatives from around the world shared their experiences and research on brain development in children with SMA.
Aim 2: Addressing Parents' Concerns & Understanding Development: The group reviewed current practices and investigated the best ways to assess neurodevelopment in children with SMA.
Aim 3: Improving Early Support & Care: There is an urgent need for recommendations on early interventions to support children's cognitive, communication, and behavioural development.
Aim 4: Planning Future Research & Collaboration: The meeting identified key gaps in knowledge and set plans for future research.

Workshop deliverables

Experts agreed that many children with SMA Type 1 who survive because of new treatments are now showing a range of developmental difficulties that can affect their speech, social skills, behaviour, and learning. They noted that this fits with recent scientific studies suggesting that low levels of the SMN protein may influence brain development even before birth and in early infancy. Because it is still unclear how current treatments affect children's development in the long term, the group agreed that more research is urgently needed. They emphasised the importance of spotting potential problems early and making sure families are quickly connected with the right specialists and therapists, while also providing strong support for the wellbeing of both children and their families. The group agreed on a basic approach for developmental screening in clinics, but recognised that more detailed diagnostic and treatment guidelines will require further research and international collaboration.

Participants of the 286th ENMC International workshop on muscle imaging.

286th ENMC International workshop: Muscle imaging: artificial intelligence, automatic segmentation and imaging data sharing in neuromuscular disease (Themed Workshop)

Background

In neuromuscular diseases (NMDs), muscles can lose volume, can be replaced by fat or be affected by inflammation. In many NMDs, these changes affect different muscles to different degrees, leading to selective and sometimes very specific patterns of muscle abnormalities. Quantitative muscle magnetic resonance imaging (qMRI) can measure muscle volume, fatty, inflammatory, necrotic or dystrophic changes objectively and non-invasively. This makes qMRI a useful imaging biomarker for diagnosing NMDs, tracking disease progression, and evaluating treatment response.

To use qMRI effectively in everyday medical practice, clinicians and researchers need consensus to identify the specific qMRI techniques to perform, accurate methods for identifying each muscle, and standardised ways to report the results. One of the biggest challenges is identifying each muscle in the MR images, known as "muscle segmentation". Recently, computer-based tools using artificial intelligence (AI) have been developed to automate this process. Nevertheless, there is no agreement yet on the best method or tool to use. Additionally, large collections of MRI scans are needed from multiple medical centres for training and testing to improve these AI tools.

Aims of the workshop

Aim 1: qMRI Image acquisition: The goal is to agree on standard practices for performing qMRI scans and storing the images.

Aim 2: qMRI Data analysis: The aim is to set standards for processing, analysing, and describing the data from these qMRI studies.

Aim 3: Automatic segmentation: The focus is to agree on standards for processing, analysing, and describing the data from automatic muscle segmentation in qMRI studies.

Aim 4: Data sharing: The objective is to build stronger collaboration between centres in Europe and around the world, to improve strategies for sharing imaging data and ensuring its long-term sustainability.

Workshop deliverables

The experts agreed on a standard procedure for performing qMRI scans to diagnose NMDs, specifically to measure fat content as an imaging biomarker and water-T2 values as an imaging biomarker of disease activity. The data from these scans will be stored in a standardised format, making it easier to compare results across different locations and studies and simplifying the analysis and sharing of qMRI data. For diagnostic purposes, segmenting individual muscles is necessary, whereas grouping muscles together may be useful for tracking disease progression.

The experts also recommend investing in machine-learning resources to implement automatic muscle segmentation in clinical practice. More research is needed to compare different automatic segmentation methods and to ensure accuracy of the segmentations for various NMDs.

Therefore, organising an international challenge to compare muscle segmentation techniques will be pursued. To aid the implementation of qMRI-based biomarkers in clinics, a standardised format for radiological reporting will be created.

287th ENMC International workshop: Harmonisation and federated analysis of myotonic dystrophy registries to model heterogeneous disease trajectories

Background

Myotonic dystrophy type 1 (DM1) is a disease that becomes worse over time and affects people in different ways. It mainly causes the muscles to become weaker, but it can also affect other parts of the body, like the heart, lungs, digestive system, and brain. It is still difficult to understand how the disease changes over time and how well different treatments work. Around the world, there are more than 25 patient databases that collect information from over 10,000 people with DM1. However, each database collects and stores information in its own way. Because of this, it is hard to combine the data.

Aims of the workshop

The aim of the workshop was to find ways to make the information in the databases easier to find, use, and share. The group also wanted to agree on what kind of information should be collected in all databases and how to safely study the data without moving it to one place. Another important goal was to understand how to measure effects of new drug treatments over a longer period of time.

Workshop deliverables

The group agreed on several concrete next steps to strengthen collaboration across databases. First, all databases should work together to create a shared, basic set of symptoms and tests that everyone collects, making it easier to compare findings and detect subtle changes in the condition. The group also identified key gaps — such as poor measurement of cognitive and digestive issues — which should be prioritised in future studies. Participants agreed to move forward with federated analysis, a method that lets researchers study data securely where it is stored, protecting privacy while enabling larger, more powerful studies that are essential for approving new treatments and securing reimbursement. It was also agreed that doctors, researchers, patients, and legal experts should jointly set research priorities, supported by an independent advisory group to guide decision-making. Finally, the meeting highlighted the need to secure long-term funding for this shared work, and several potential funding models were outlined for follow-up.

Participants of the 287th ENMC International workshop on myotonic dystrophy registries.

Participants of the 288th ENMC International workshop on gastrointestinal symptoms in myotonic dystrophy.

288th ENMC International workshop: Towards better diagnosing, understanding and treating gastrointestinal symptoms in myotonic dystrophy

Background

In myotonic dystrophy type 1 (DM1) and type 2 (DM2), not only muscles but also other parts of the body can be affected. Gastrointestinal problems — such as abdominal pain, constipation, and diarrhoea — are common. It is not yet known what causes these symptoms or why they vary significantly from person to person. Currently, there is little focus on or knowledge about these problems, which makes it difficult to recognise and treat these symptoms.

Aims of the workshop

Aim 1: Understand how common gastrointestinal problems are in DM, what they are like, how serious they can be, and how they affect daily life.
Aim 2: Explore what causes these problems and how to detect and treat them.
Aim 3: Develop a guideline to help doctors include gastrointestinal problems as part of regular care for people with DM.
Aim 4: Create a practical and straightforward questionnaire to monitor symptoms over time and evaluate the effects of treatment(s).

Workshop deliverables

Given the high prevalence and burden of GI symptoms in children and adults with DM1 and DM2, systematic screening, diagnostic evaluation, and tailored management are warranted.
This workshop highlighted the broad spectrum of GI manifestations and the need for increased clinical awareness. Expert consensus formed the basis for preliminary recommendations to optimise current care, enhance the design of clinical trials, and guide future directions in animal research. In collaboration with patient representatives, a practical one-page questionnaire was developed to facilitate symptom recognition in routine care. While this workshop represents an important initial step, ongoing efforts are needed to build consensus and ultimately create clinical guidelines for the management of GI symptoms in individuals with DM, as well as develop animal models to identify key drivers of GI symptoms. Improved understanding and management of GI symptoms in DM have the potential to significantly enhance patient well-being and quality of life; it is time this domain receives the clinical and research attention it deserves.

289th ENMC International workshop: Assessing and managing emerging AAV related toxicities after gene therapy for neuromuscular disorders

Background

Adeno-associated viral (AAV) mediated gene therapy is a very powerful technology that has the potential to significantly improve quality of life of patients affected by a variety of neuromuscular conditions. Several products have already been approved in gene therapy in general and others are at different stages of clinical development. Several publications have highlighted the benefits of approved and emerging therapies in managing neuromuscular disorders. Nevertheless, a range of severe adverse events have been observed in clinical trials and have emerged in the real-world use of these novel products. These included several complications affecting the blood, the heart, and the liver, some of which were fatal while others resolved with no long-term complications.

Aims of the workshop

In order to continue the development of these products and improve their risk/benefit profile, the workshop was devoted to openly and collaboratively review recent findings related to those severe adverse events. Four dedicated working groups collaborated in the months leading up to the meeting with several teleconferences to prepare the groundwork for the workshop. These four working groups focused on:

1. Anti-transgene adverse events;
2. Thrombotic microangiopathy;
3. Liver related adverse events;
4. Cardiac related adverse events.

Workshop deliverables

The discussion about the four topics included learnings about their spectrum of manifestations and causation, the role of different research models in helping to understand the processes that causes them, and the potential effectiveness of existing treatments that work on the immune system in preventing or mitigating these adverse events. Novel and emerging manifestations of toxicity related to AAV gene therapy in the human were also reported, and the effect of different therapeutic modalities discussed. The last part of the meeting addressed the need to revise and share standard operating procedures and clinical guidelines on collecting data around these events. Those efforts will allow better assessment of whether each individual is a good candidate for AAV treatment and their personal level of risk before receiving an AAV gene therapy. Most importantly the value of openly

Participants of the 289th ENMC International workshop on AAV related toxicities after gene therapy.

Organisers of the 289th ENMC International workshop. From left to right: Dr Rotem Orbach (USA), Prof. Carsten Bönnemann (USA), Prof. Hildegard Büning (Germany), Prof. Francesco Muntoni (UK), Dr Rebeca Gil (UK).

sharing and comparing data on these emerging challenges associated with AAV mediated gene therapy on a pre-competitive level was recognised and strategies for effective data collection and sharing were discussed.

Multiple future collaborative options were agreed, ranging from registries of patients treated with these therapies to future working groups and meetings to continue the refinement of protocols for monitoring and management of the more common adverse events. One of the key outcomes from the meeting was the decision to establish a collaborative framework to better understand the implications of using AAV as a therapeutic vector in humans. This framework will support the development of thoughtful, patient-centred strategies to address the inherent challenges and potential toxicities of AAV therapies, helping ensure that the benefits of this still-emerging but potentially transformative approach can be fully realised.

290th ENMC International workshop: Developing standards of care for patients with Becker Muscular Dystrophy (BMD)

Background

Becker muscular dystrophy (BMD) is an X-linked recessive neuromuscular disorder caused by mutations in the dystrophin (DMD) gene, which results in the expression of abnormal and/or quantitatively defective dystrophin protein in skeletal muscle fibres. Traditionally, BMD has been treated as a separate condition rather than part of a spectrum of dystrophinopathies, excluding BMD patients from clinical trials, the development of outcome measures, biomarkers, and care standards.

Aims of the workshop

The aims of the workshop were to review current clinical practice regarding diagnosis and care of patients with BMD, identify challenges and gaps in knowledge, and outline priorities for international clinical recommendations. Surveys tailored to patients and clinicians, developed specifically for the workshop, were analysed, and published Italian and French recommendations were reviewed.

Organisers and Early Career Researchers (ECR) of the 290th ENMC International workshop. From left to right: Prof. Ros Quinlivan (UK), Prof. Elena Pegoraro (Italy), Dr Pietro Riguzzi (ECR, UK), Dr Nienke van der Velde (ECR, Netherlands), Prof. Michela Guglieri (UK), Dr Erik Niks (Netherlands).

Workshop deliverables

Participants discussed ways to improve diagnostic pathways. The need for a multidisciplinary approach was highlighted. Muscle MRI in BMD shows promise as a marker of disease progression in research studies, but its implementation needs further development and standardisation. There is still a lack of evidence to guide clinical care in several key areas, including pain management and the use of steroids. Exercise was discussed as generally safe and beneficial, but programmes need to be designed with specialist input.

Heart involvement remains a key aspect of concern in BMD care. Experts discussed the importance of early heart scans, including echoes or MRI, and early treatment. Respiratory involvement is uncommon in BMD, but still requires regular monitoring at key stages, especially in patients who are no longer able to walk or move independently. Bone health was discussed, especially the importance of regularly checking and supplementing vitamin D. Anaesthetic risk was discussed at length as proper precautions and careful selection of anaesthetic drugs are required when patients with BMD undergo general anaesthesia due to potential complications. There is a need for more education of patients, families, and healthcare professionals regarding this aspect of care.

Brain and mental health issues in BMD are increasingly recognised at all ages. Assessments of brain function and access to psychological support were identified as important, currently unmet needs. When psychiatric medication is needed, usual treatment guidelines should be followed. Extra support from the multidisciplinary team might be needed, especially by consulting with the heart specialist.

4.3 Participants at ENMC workshops in 2025

The ENMC strives for a wide range of expertise and experience in its workshop participants to ensure that a broad consensus can be reached at the meetings by having all relevant stakeholders around the table.

For each workshop that took place in 2025, the numbers of different stakeholders are shown in the table below.

No	Workshop Title	Participants	Clinicians	Basic researchers	Translationel researchers	Patients	Patient representatives	Early-Career researchers	Industry	Regulatory	Other*
283	Establishing expert care recommendations for LAMA2-RDs: a prototype for the development of congenital muscular dystrophy subtype-specific care guidelines.	24	16	1	2	1	2	2			
284	Cognitive and behavioral abnormalities in pediatric DM1 – what should we measure in preparation for clinical trials?	27	10	4	2		5	2	3	1	
285	SMN-related neurodevelopmental disorder: type 1 Spinal Muscular Atrophy and the brain.	28	16	2	5		2	3			
286	Muscle imaging: artificial intelligence, automatic segmentation and imaging data sharing in neuromuscular disease.	18	7	6	2			2			1
287	Harmonisation and federated analysis of Myotonic Dystrophy registries to model heterogeneous disease trajectories.	21	5	1	2		4	2	2	1	4
288	Towards better diagnosing, understanding and treating gastrointestinal symptoms in myotonic dystrophy.	26	11	3	2		4	3			3
289	Assessing and managing emerging AAV related toxicities after gene therapy for neuromuscular disorders.	38	14	4	3		4	2	11		
290	Developing Standards of Care for patients with Becker Muscular Dystrophy (BMD).	23	17		1	1	2	2			
Average of 9 workshops (n)		**26**									
%		100%	47%	10%	9%	1%	11%	9%	8%	1%	4%
Total of 9 workshops (n)		**205**	**96**	**21**	**19**	**2**	**23**	**18**	**16**	**2**	**8**

**Data scientists and paramedics.*

Clinicians, translational researchers and basic researchers formed the majority of the participants (66%), with an average of 47% clinicians, 10% basic researchers and 9% translational researchers per meeting, which reflects the predominantly clinically orientated nature of the workshops held in 2025.

Connecting basic researchers with clinicians to bridge the lab and the clinic and bring the fundamental science closer to the clinic is one of the aims of the ENMC.

Through the ENMC Patient Participation Programme we aim to ensure that at least 10% of the participants of each workshop are persons affected by a neuromuscular condition, parents or advocates of these patients and/or representatives from a disease-specific patient or funding organisation. In 2025, these two groups made up 12% of total participants; the patients' voice was well represented.

On average, two Early-Career researchers attended each ENMC workshop this year. ENMC supports the integration of the next generation of clinicians and basic scientists in established neuromuscular networks via its Early-Career Programme and Mid-Career Mentoring Programme.

In five workshops, where it was relevant, workshop participants included data scientists, paramedics and representatives from pharmaceutical companies and the European Medicines Agency.

New numbers to be proud of in 2025

Patients and patient representatives
This year we welcomed two patients and twenty-three patientrepresentatives (parents, patient associations, patient advocates) to ENMC workshops. They all gave a presentation, asked questions and throughout in the discussions, which was very helpful for the researchers and clinicians in the workshops to learn the patients' needs and interests.

Researchers
We held nine workshops in 2025, with the attendance of 21 basic researchers, 19 translational researchers and 96 clinicians, respectively 10%, 9% and 47% of the total participants. The Early-Career Programme enabled 18 early career researchers and clinicians to attend the ENMC workshops and promote their entry in the neuromuscular network.

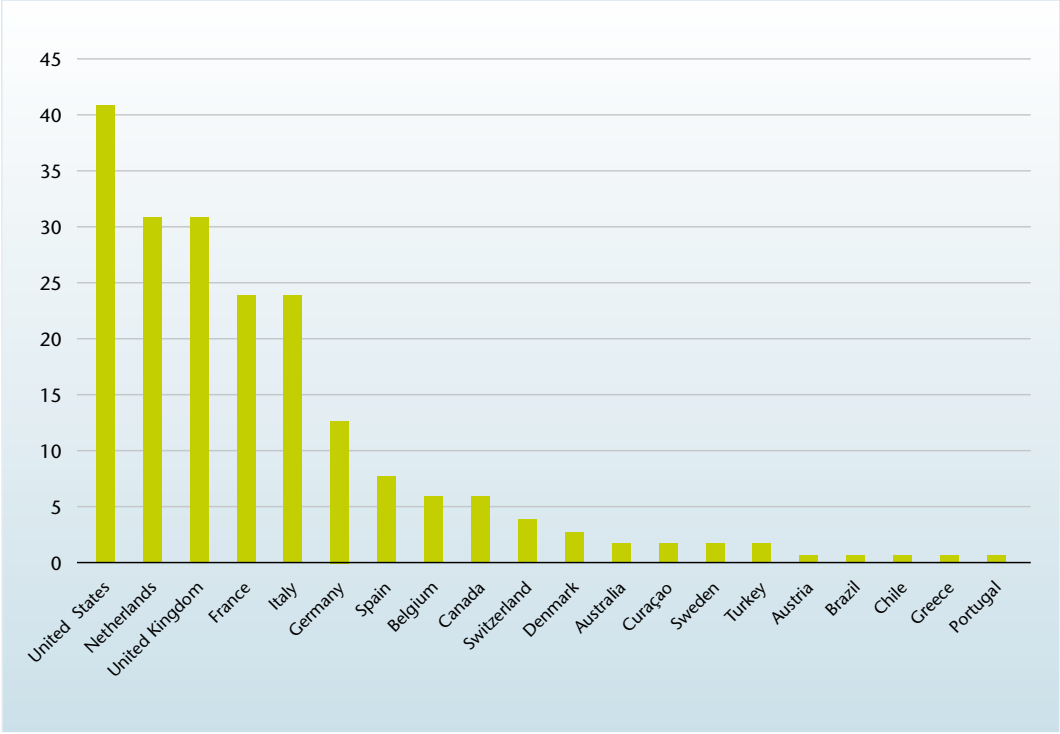
Sponsors
In 2025, the ENMC was sponsored by eight full partners and five associated partners. The ENMC Company Forum supported our activities through the contributions of eight pharmaceutical companies. We are very thankful for the support from all these partners and acknowledge them with gratitude. In addition to these permanent sponsors, we also would like to thank the great contributions of different organisations that co-sponsored specific workshops in 2025.

4.4 Countries represented in ENMC workshops in 2025

One of the key criteria for a workshop approval by the ENMC is the geographical distribution of the participants. The ENMC is convinced that a wide coverage of countries in the workshops is important to make sure that broad consensus is reached at ENMC workshops and that this consensus is shared widely. This will help to start international research collaborations, ensure the standardisation of healthcare provision for people affected by a neuromuscular condition, and improve the quality of diagnosis and treatment for patients worldwide.

In 2025, 20 countries from all over the world were represented at the ENMC workshops (see table below), with a predominance of the ENMC full member countries (The Netherlands, United Kingdom, France, Italy, Germany, Switzerland and Denmark) and some non-member European countries like Spain, Belgium, Sweden and Greece. Non-European countries like USA, Canada, Australia, Curaçao, Turkey, Brazil and Chile were also represented by individual researchers and clinicians at the nine workshops in 2025.

Total number of participants per country in ENMC workshops 2025



5 Spotlight: Stichting Voor Sara and ENMC Workshop on LAMA2-RD - Interview with Bram Verbrugge

BRAM VERBRUGGE is the founder and chair of **Stichting Voor Sara**, a Dutch patient organisation dedicated to children and adults living with the rare neuromuscular disease LAMA2-RD (also known as LAMA2-MD or MDC1A). Bram became involved in the neuromuscular community through his role as the father of his daughter Sara, who herself lives with this incurable muscle disease. Drawing on his personal experiences, he combines the perspective of a caregiver with that of a passionate advocate for improved care, stronger collaboration between experts, and accelerated research into treatments. His voice serves as an important bridge between patients, families, and international experts.

STICHTING VOOR SARA was founded in late 2016 by the parents of Sara Verbrugge shortly after she was diagnosed with LAMA2-RD, a severe and ultra-rare inherited muscle disease for which there was, at the time, very limited knowledge about optimal care and effective therapies. Currently, there is no treatment available for this disease. The severity of LAMA2-RD varies greatly between patients; however, the most severe forms are associated with a significantly reduced life expectancy.

VOOR SARA

The mission of **Voor Sara** is to:

- Achieve better care and effective treatments for patients with LAMA2-RD and other rare neuromuscular diseases.
- Increase awareness among healthcare professionals, researchers, policymakers, and the general public.
- Fund, support, and accelerate research, both in the Netherlands (including at Maastricht University and RadboudUMC) and internationally.
- Connect patients, families, and experts through meetings, conferences, and knowledge sharing.

Over the years, Voor Sara has grown into an international organisation that collaborates closely with universities, medical centres, and other patient organisations.

How did you get involved with ENMC?

I first became involved with the European Neuromuscular Centre (ENMC) when *Voor Sara* requested a workshop dedicated to LAMA2-RD in 2019. The request was declined. We were disappointed, but in retrospect, it is understandable. There were hardly any publications about our daughter's muscle disease. There was almost no information to be found. We got to work and, fortunately, the American foundation Cure CMD was already active. A lot has changed in recent years. So in 2024 we tried for the second time and we co-initiated a proposal for an international clinical workshop focused on LAMA2-RD. Together with Cure CMD and leading clinicians from around the world, we submitted the application — and were grateful when ENMC approved it for January 2025 in the Netherlands. This initiative grew directly out of our desire to bring international expertise together and improve care for patients and families facing this condition.

What has been your experience of organising/participating in an ENMC workshop?

The organisation is a considerable undertaking, and the preparation is at least as important as the workshop itself. We sought to compile a comprehensive list of experts. It was also necessary to find the right balance between our ambitious plans and a realistic objective for a single weekend. Fortunately, thanks to the collaboration with the ENMC and the organisers of this workshop, we were able to achieve this successfully.

Participating in the ENMC workshop was an incredibly enriching experience. Bringing together experts, clinicians, and caregivers created an atmosphere of genuine collaboration and mutual respect. It was inspiring to hear the depth of knowledge and commitment from specialists who had dedicated their careers to neuromuscular diseases. At the same time, it was equally powerful to integrate patient and caregiver perspectives. I felt that ENMC provided a uniquely inclusive platform where all voices mattered — not just scientific, but human.

What were your goals for the ENMC workshop on LAMA2-RD?

Our core goals for the ENMC workshop were to:

- Summarise existing evidence and expert knowledge on LAMA2-RD clinical care.
- Identify critical knowledge gaps in care and management.
- Formulate initial expert care recommendations.
- Establish a method and process for developing official clinical care guidelines for LAMA2-RD.
- Lay the foundation for a living document that can grow as evidence evolves.

  We also aimed to ensure that the perspectives of patients and families were interwoven into these goals from the very beginning.

What was the effect/results of the ENMC workshop on LAMA2-RD for your organisation *Voor Sara*?

Short-Term Impact

The workshop catalysed key agreements on several aspects of clinical care and highlighted where evidence is lacking. It also fostered ongoing collaboration between multidisciplinary specialists, researchers, and advocacy partners. It was the starting point for drawing up care guidelines aimed at improving care for all patients. The ENMC workshop has already led to a publication about the workshop and our plan to establish the first care guideline for LAMA2-RD, an important milestone in standardising care.

Bram Verbrugge with daughter Sara.

Bram Verbrugge presented at the ENMC Workshop on LAMA2-RD.

Long-Term Impact

This guideline — rooted in the workshop's discussions — will be a major step toward consistency in clinical practice worldwide, helping patients receive comparable quality of care regardless of location. The guideline is designed to remain dynamic and updated as new knowledge emerges. For *Voor Sara*, the workshop strengthened our strategic network within the international neuromuscular community and has directly contributed to accelerating research momentum and aligning stakeholders towards better outcomes for patients. We are proud that, as a small foundation, we can make such a big impact in this way, thanks to these partnerships.

Why do you think it's so important to have patient/patient representation in a workshop?

Patient and caregiver representation is essential because families live with LAMA2-RD every single day. They experience first-hand what works in real life and what doesn't — insights that simply can't be fully captured by clinical data or scientific literature alone. When patients and caregivers are part of the conversation, it helps ensure that discussions focus on what truly matters most to those affected, rather than only on theoretical or academic priorities.

Equally important is the sense of empowerment and trust this creates. When patients feel genuinely heard and taken seriously, it strengthens the relationship between the patient community and the scientific and medical world. I also strongly believe that direct contact between experts and patients is incredibly valuable. Meeting the people behind the disease — hearing their stories and understanding the real-life impact of their work — can be a powerful source of motivation for clinicians and researchers. Ultimately, involving patient voices leads to better outcomes: care recommendations become more relevant, more practical, and more likely to be adopted in everyday clinical practice.

What ambitions do you see for the ENMC in the near future?

I believe ENMC is uniquely positioned to continue strengthening inclusive, multidisciplinary collaborations that bring together scientific expertise and lived experience. By creating space for open dialogue between clinicians, researchers, and patient representatives, ENMC helps ensure that discussions lead to meaningful and relevant outcomes.
In the near future, I see an important role for ENMC in further promoting the development of clinical care guidelines, especially for rare diseases like LAMA2-RD, where standardised care pathways are often lacking. In addition, supporting meaningful patient participation — in research, clinical trial design, and guideline development — will remain essential.

Finally, ENMC has the opportunity to serve as a blueprint for other rare disease communities.
The LAMA2-RD workshop showed how collaborative, patient-inclusive workshops can accelerate progress, and this model could be successfully applied to many other rare conditions.

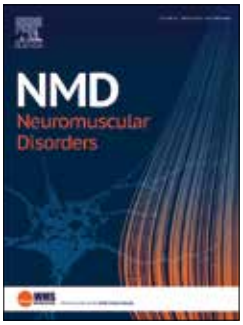
The partnership between ENMC and *Voor Sara* exemplifies the power of collaboration between patients, clinicians, researchers, and advocacy groups. Through initiatives like the LAMA2-RD workshop, we are not only shaping better care today — we are laying the foundation for a future where rare disease communities have the standards, knowledge, and shared understanding they urgently need.

6 Creating global awareness about ENMC workshops

6.1 Publication and dissemination of workshop outcomes

Informing patients and their families about the achievements of ENMC workshops is a key priority of the ENMC. For this purpose, a workshop lay report in English is written by workshop participants and published on the ENMC website within two weeks after the workshop. Lay reports are then translated into many different languages to increase their accessibility for people worldwide. The European partner organisations and other co-sponsors help to disseminate these translated lay reports via their local patient networks and the ENMC creates awareness on social media (LinkedIn: ENMC Non-profit group) during the workshops and whenever lay reports are published on its website. The ENMC maintains an online archive of all workshops organised since 2000, which provides access for the general public to the outcome of the workshops over the years: **https://www.enmc.org/reports/lay-reports/**

Follow us, like, and repost!



Research community

Researchers, clinicians and healthcare providers who are active in the research field of rare neuromuscular disorders need to be able to read about the scientific results of ENMC workshops. Therefore, it is mandatory that workshop organisers submit a full workshop report to the journal Neuromuscular Disorders within six months of the workshop. Since 2021, Early-Career Researchers who made a significant contributions to the organisation of the workshop and the writing of the lay and full report, could become co-authors on the full report.

Citation score 124%

ENMC-workshop derived publications are cited 24% more than average. In bibliometric terms this means that they have a "high impact" in the NMD research field.

6.2 International conferences in 2025

ENMC ambassadors have attended the following international congresses:

- Congress of the Medical Scientific Advisory Board of the German Muscular Dystrophy Society DGM e.V., Giessen, Germany (19-21 March 2025).
- 18th UK Neuromuscular Translational Research Conference 2025, Newcastle upon Tyne, United Kingdom (15-16 April 2025).
- Peripheral Nerve Society Annual Meeting (PNS), Edinburgh, United Kingdom (17-20 May 2025).
- European Academy of Neurology (EAN), Helsinki, Finland (21-24 June 2025).
- 16th European Paediatric Neurology Society Congress, Munich, Germany (8-12 July 2025)
- World Muscle Society Congress, Vienna, Austria (7-11 October 2025).
- 2nd European CMT Specialists Conference, Antwerp, Belgium (24-25 October 2025).

The ENMC was present at the Congress of the Medical Scientific Advisory Board of the German Muscular Dystrophy Society DGM e.V. with a booth. From left to right at the DGM booth: Silke Schlüter, Petra Hatzinger, Wilma Hinloopen (ENMC), Patricia van Dongen (ENMC) and Katharina Kohnen.

7 The ENMC Mid-Career Mentoring Programme

This programme has been developed for people who seek mentoring to acquire skills on their way toward becoming independent researchers and/or potential future leaders in the NMD field. These individuals typically are in the stage in their careers where they are developing their own research plans and have a proven track record in the neuromuscular field. They have established research teams and collaborative networks. The guidelines and the mentee and mentor forms can be found on the ENMC website:

Mid-Career Mentoring Programme

https://www.enmc.org/mid-career-mentoring-programme/introduction/

Deadline to apply is **July 1st** each year.

In the fifth call for the ENMC Mid-Career Mentoring Programme with the deadline July 2025, we received eight applications! Following interviews with each of the candidates, the ENMC Mentoring Programme Committee presented their recommendations to the Research- and Executive Committees. We are very happy to announce that six mentee candidates were selected for the programme.

Mentees 2025

DR TERESA GERHALTER is a senior scientist at Department of Neurology at the Medical University of Graz, Austria.

Teresa's mid-career mentors are Prof. Cynthia Gagnon (Canada) and Prof. Maria Pennuto (Italy).

DR CLARA GONTIJO CAMELO is a paediatric neurologist at the Faculty of Medicine of the University of São Paulo, Brazil.

Clara's mid-career mentors are Prof. Heinz Jungbluth (United Kingdom) and Prof. Ulrike Schara-Schmidt (Germany).

DR FELIX KLEEFELD is a neurologist at the BG University Hospital Bergmannsheil in Bochum, Germany.

Felix's mid-career mentors are Dr Andrew Mammen (United States) and Prof. Bert Smeets (The Netherlands).

DR MAURO MONFORTE is a neurologist and group leader at the Fondazione Policlinico Universitario A. Gemelli IRCCS, and a researcher at the Catholic University of the Sacred Heart in Rome, Italy.

Mauro's mid-career mentors are Prof. Hanns Lochmüller (Canada) and Prof. Kathryn Wagner (United States).

DR KARLIEN MUL is a neurologist at Radboud university medical centre, Nijmegen, The Netherlands.

Karlien's mid-career mentors are Dr Thomas Meier (Switzerland) and Prof. Volker Straub (United Kingdom).

DR RHONDA TAYLOR is a researcher at the Harry Perkins Institute of Medical Research, Perth, Australia.

Rhonda's mid-career mentors are Prof. Annamaria De Luca (Italy) and Prof. Eric Hoffman (United States).

8 Resources and financial management in 2025

Financial summary 2025

Annual accounts for the year 2025 were compiled in accordance with Guideline C1 for the reporting of small-sized non-profit organisations as published by the Dutch Accounting Standards Board. The financial accounts are drawn up in Euros.

In the summary table below, the overall income and expenditure over the year 2025 are shown in comparison with the figures for the previous financial year 2024.

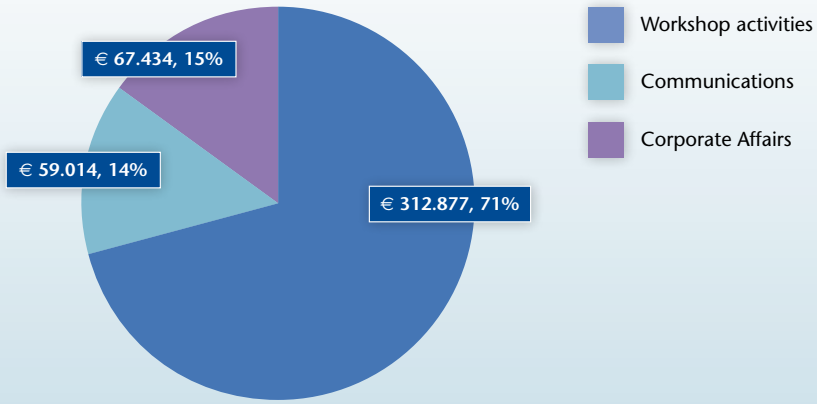
Statement of income and expenses for the year 2025 in Euros (€)		
	2025	2024
INCOME		
Full Partner contributions	242.550	231.000
Associated Partner contributions	25.000	25.000
Company Forum contributions	127.257	146.556
Other contributions	41.983	64.990
Total income	**436.790**	**467.546**
EXPENSES		
Personnel expenses	221.916	211.202
Rental expenses	11.868	11.893
Activity (workshop) expenses	143.744	223.367
Organisational expenses	61.797	51.091
Total operating expenses	**439.325**	**497.553**
Operating result	– 2.535	– 30.007
Interest income	6.783	8.148
Net result	**4.248**	**– 21.859**
APPROPRIATION OF RESULTS		
Transitional reserve - release	–	– 114.278
Global Travel Fund - allocation	–	114.278
Global Travel Fund - used	– 27.000	– 30.000
Other free reserves	31.248	8.141
CASH AT BANKS ON 31 DECEMBER	**665.086**	**637.208**

The distribution of income from the different ENMC supporters and the distribution of costs over the key accounts: workshop activities, corporate affairs and communications, are provided in the two diagrams.

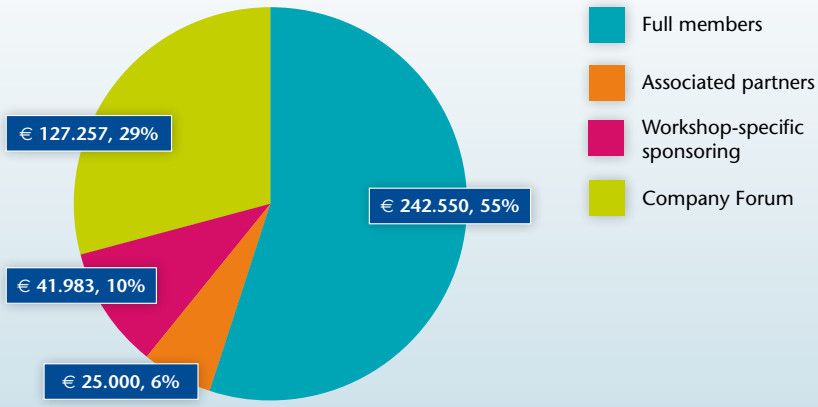
Opinion of the auditors

The independent accountants have verified and approved the annual accounts. For a full PDF version of the report of the annual accounts for 2025, please visit the ENMC website: **https://www.enmc.org/about-us/annual-report/**

Distribution of costs **2025**



Distribution of income **2025**



9 Governance in 2025

The European Neuromuscular Centre was founded as a non-profit organisation on 24 November 1992 under Dutch law. The foundation is supported by financial contributions of European patient organisations for neuromuscular disorders and many other related organisations. The statutory location is in Baarn, The Netherlands, in the building of Spierziekten Nederland.

9.1 The ENMC Executive Committee

The ENMC is governed by an Executive Committee consisting of representatives of ENMC partner organisations.

Composition of the ENMC Executive Committee on 31 December 2025
Dr K. Adcock (vice-Chair, United Kingdom)
Dr D. Baldessari (Italy)
Dr S. van den Berge (The Netherlands)
Mr H. Ib Jørgensen (Denmark)
Dr I. Meijer (Chair, The Netherlands)
Dr A. Méjat (France)
Prof. U. Schara-Schmidt (Germany)
Dr R. Willmann (Switzerland)

9.2 The ENMC Research Committee

The ENMC Research Committee is responsible for reviewing the scientific content and quality of the workshop applications and advises the Executive Committee on awarding the grants for ENMC workshops.

Composition of the ENMC Research Committee on 31 December 2025
Dr A. Buj-Bello (France)
Prof. K. Claeys (Belgium)
Prof. E. Gomes (Portugal)
Prof. C. Handberg (Denmark)
Prof. C. Kornblum (Germany)
Prof. C. Ottenheijm (The Netherlands)
Dr C. Paradas (Spain)
Dr D. Pareyson (Italy)
Prof. R. Quinlivan (Chair, United Kingdom)
Prof. G. Tasca (United Kingdom)
Prof. M. Weber (Switzerland)

ENMC Executive Committee in April 2025.

ENMC Research Committee meeting in November 2025.

9.3 The ENMC Office

The office takes care of the daily business of the ENMC.

ENMC Office staff on 31 December 2025
Ms P. van Dongen (Programme Manager)
Ms W. Hinloopen (Operational Manager)
Ms T. van Esch (Freelance Workshop Assistant)

ENMC office staff, Patricia van Dongen and Wilma Hinloopen.

9.4 Transfer of the Spierziekten Nederland representative

After representing Spierziekten Nederland at the ENMC Executive Committee for 10 years (from 2015-2025), Dr Ingeborg Meijer passed her task to Dr Anja Horemans in November 2025.
From the very beginning, Ingeborg's contributions to the mentoring committee have been invaluable — from helping to establish the scheme to interviewing candidates for the ENMC Mid-Career Mentoring Programme. Her pragmatic approach kept everybody grounded and focused.
What stands out most is her strong commitment to the societal impact of science. She did more than speak about impact; she introduced practical tools, methods, and fresh perspectives that challenged our traditionally science-centered thinking and encouraged the ENMC office to view its work through a broader lens.
At the 235th ENMC workshop on the position of neuromuscular patients in Shared Decision Making in Milan, Italy (2018), she skillfully coordinated discussions, gathered and prioritized input, and reorganized groups for a second round before the final presentations.
In evaluating the impact of ENMC workshops, she introduced innovative ways to quantify collaborations and track awareness of workshop papers, exposing ENMC to approaches that had not previously been considered.

We extend our deepest gratitude to Ingeborg for her insight, creativity, unwavering dedication, and her ability to make even complex tasks achievable — and enjoyable — as well as for her lasting commitment to ENMC over the years.

Dr Ingeborg Meijer (right) handing over the gavel to Dr Kate Adcock, Director of Research and Innovation at Muscular Dystrophy UK (left), who will be the chair of the ENMC Executive Committee during the EC meeting in November 2025.

Dr Anja Horemans

Dr Anja Horemans is senior manager at Spierziekten Nederland, the Dutch patient organisation of people with neuromuscular diseases. She obtained her PhD in biochemistry at Utrecht University's Faculty of Veterinary Medicine. After her doctoral research, she joined Spierziekten Nederland, where she and her team work to improve the quality of care and treatment for people with neuromuscular diseases in the Netherlands.

Within the field of care, she has contributed to the development of clinical guidelines and practical information for healthcare professionals. Currently, she is involved in the development of a national neuromuscular care network. She and her team also contribute to neuromuscular research from the patient perspective. In recent years, her work has increasingly focused on access to and reimbursement of new, often costly, therapies for neuromuscular diseases.

The ENMC welcomes her to the Executive Committee and looks forward to a great collaboration.

Dr Anja Horemans

10 A special thank-you to all our partners and supporters

It is thanks to the continuous support of the eight European patient organisations that the ENMC can facilitate and organise, on average, eight workshops per year. With support from additional partner organisations, such as condition-specific associations, associated partners and members of the ENMC Company Forum, we are also able to invite participants from non-ENMC countries and facilitate the attendance of Early-Career Researchers, patients and patient representatives.

ENMC full partners

AFM TÉLÉTHON
INNOVER POUR GUÉRIR

Deutsche Gesellschaft für Muskelkranke e.V. DGM

FONDAZIONE Telethon

fsrmm
fondation suisse de recherche sur les maladies musculaires
fondazione svizzera per la ricerca sulle malattie muscolari
schweiz. stiftung für die erforschung der muskelkrankheiten

MUSCULAR DYSTROPHY UK
OUR MUSCLES MATTER

MUSKELSVINDFONDEN

Prinses Beatrix Spierfonds

SPIERZIEKTEN NEDERLAND

ENMC associated partners

Members of the Company Forum

Workshop-specific sponsors in 2025

11 Looking to 2026 and beyond

11.1 Workshops in 2026

Six ENMC workshops are scheduled to take place in the first half year of 2026 and at least three further workshops are planned later in the year (see table below). Two review rounds for workshop applications are scheduled in 2026: one in the spring (submission deadline 1 March 2026) and one in the autumn (submission deadline 1 September 2026). The workshops that are selected at these review rounds will all be planned for Q4 2026 or 2027. For updates please visit the ENMC website: **https://www.enmc.org/workshops/upcoming-workshops/**

Preliminary ENMC programme 2026

Workshop no. and date	Topic	Workshop leaders
Workshop no. 291 16-18 January 2026	Optimizing collaborative models in neuromuscular diseases: the role of ontologies and artificial intelligence (THEMED WORKSHOP).	Prof. Cynthia Gagnon, Prof. Anita Burgun
Workshop no. 292 23-25 January 2026	Best practices after positive Spinal Muscular Atrophy newborn screening.	Prof. Liesbeth De Waele, Dr Monika Gos, Marie-Christine Ouillade, Prof. Eduardo Tizzano
Workshop no. 293 6-8 March 2026	Immune checkpoint inhibitors myotoxicity: diagnostic criteria and therapeutic consensus.	Prof. Yves Allenbach, Dr Iago Pinal Fernandez, Prof. Joe-Elie Salem
Workshop no. 294 27-29 March 2026	Diagnosis and management of paraproteinemic myopathies focusing on sporadic late onset nemaline myopathy (SLONM) and light chain (AL) amyloid myopathy.	Prof. Teerin Liewluck, Dr Felix Kleefeld, Dr Matteo Garibaldi, Prof. Pascal Laforêt
Workshop no. 295 1-3 May 2026	Harmonizing clinical monitoring in FSHD: clinical outcome measure, patient perspectives, and digital innovations for the upcoming era of drug therapies.	Dr Katy Eichinger, Dr Enrico Bugiardini, Prof. Benedikt Schoser
Workshop no. 296 19-21 June 2026	Collagen VI fundamental insights and preclinical innovations: advancing towards translation for COL6-RD.	Dr Valérie Allamand, Prof. Carsten Bönnemann, Dr Cecilia Jimenez-Mallebrera
Workshop no. 297 30 October-1 November 2026	A mechanism-based nomenclature and classification in Myofibrillar Myopathies.	Prof. Kristl Claeys, Prof. Giorgio Tasca, Dr Lorenzo Maggi, Prof. Chris Weihl
Workshop no. 298 13-15 November 2026	Clinical care guidelines, outcome measures, and clinical trial readiness in Oculopharyngeal Muscular Dystrophy (OPMD).	Dr Jodi Warman-Chardon, Prof. Nicol Voermans, Prof. Giorgio Tasca, Dr Teresinha Evangelista
Workshop no. 299 11-13 December 2026	Recommended standards for muscle pathology: from the EURO-NMD experience to a uniform and concise muscle biopsy reporting tool, and digital pathology implementation worldwide.	Prof. Edoardo Malfatti, Prof. Werner Stenzel, Dr Teresinha Evangelista, Prof. Kristl Claeys

11.2 International conferences in 2026

ENMC ambassadors will attend the following international congresses.

- 5th International Congress on Spinal Muscular Atrophy, Budapest, Hungary (12-14 March 2026).
- 19th UK Neuromuscular Translational Research Conference 2025, Cambridge, United Kingdom (17-18 April 2026).
- Peripheral Nerve Society Annual Meeting (PNS), Maastricht, Netherlands (13-16 June 2026).
- European Academy of Neurology (EAN), Geneva, Switzerland (27-30 June 2026).
- 19th International Congress on Neuromuscular Diseases, Florence, Italy (7-11 July 2026).
- World Muscle Society Congress, Hiroshima, Japan (29 September-3 October 2026).

11.3 Budget for 2026

This table presents the budget forecast for 2026 as of 31 December 2025.

Budget 2026 in Euros (€)	Actuals 2025	Budget 2026
INCOME		
Full Partner contributions	242.550	242.550
Associated Partner contributions	25.000	30.000
Company Forum contributions	127.257	120.000
Other contributions	41.983	40.000
Total income	**436.790**	**432.550**
EXPENSES		
Personnel expenses	221.916	232.462
Rental expenses	11.868	12.000
Activity (workshop) expenses	143.744	195.000
Organisational expenses	61.797	54.000
Total operating expenses	**439.325**	**493.462**
Operational result	– **2.535**	– 60.912
Interest income	6.783	6.000
NET RESULT	**4.248**	**– 54.912**

The ENMC was present at the World Muscle Society congress with a booth. From left to right: Wilma Hinloopen, Prof. Ros Quinlivan (Research Director), Prof. Ulrike Schara-Schmidt (EC member) and Patricia van Dongen.

Colophon

Text editors
Kate Adcock
Simone van den Berge
Patricia van Dongen
Wilma Hinloopen
Ingeborg Meijer

Photographers and illustrators
ENMC

Design and lay-out
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Printing
Year of printing 2026, printing by DrukwerkDeal

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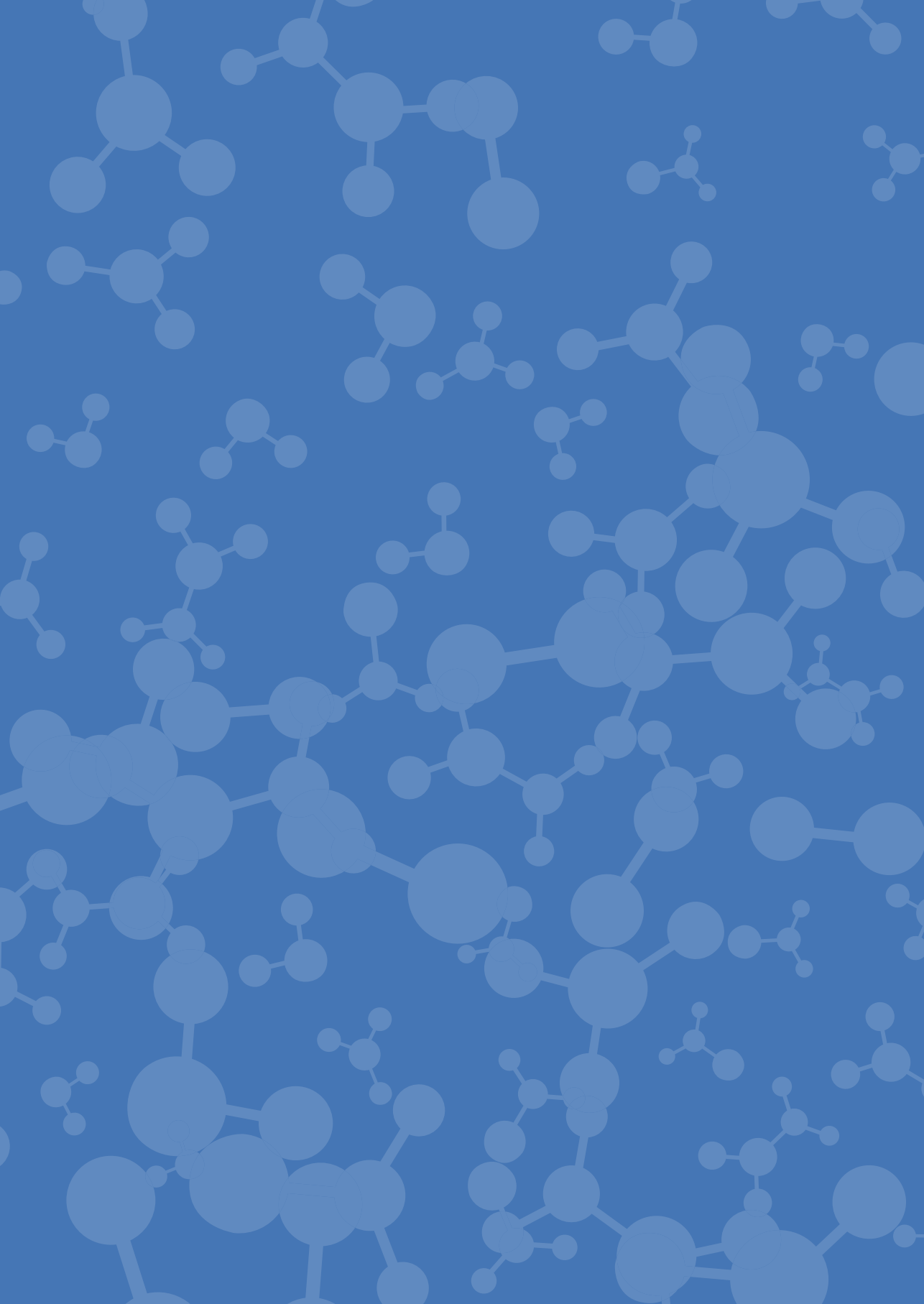

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