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Original research article

Building capacity for patient-engagement in neuromuscular disease research: A network project

Journal of Neuromuscular Diseases
1–12
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DOI: 10.1177/22143602251405819
journals.sagepub.com/home/jnd

Patricia Mortenson[1,2,3], Homira Osman[1,4], Erin Beattie[1], Corinne Kagan[1], Victoria Larocca[1,4], Claudia Maltais[1], Linda Niksic[1], Margo Thompson[1] and Kathryn Selby[1,5,6]

Abstract

Patient-oriented research is increasingly recognized as an important methodology in health sciences. Benefits of patient engagement include aligning research priorities to those living with health conditions, developing better recruitment strategies and protocols, and integrating findings more meaningfully. However, for research teams to work well with patient-partners, training for all stakeholders is needed. While training exists, none consider the uniqueness of the neuromuscular disease experience. To address this gap, our team of researchers, clinicians, and patient-partners collaborated to increase the capacity for patient-engagement in neuromuscular disease research. Our methods included: 1) conducting a landscape of available resources, and 2) using adult education principles and a backwards design process to develop unique training modules. The result is an online platform with three modules focusing on the neuromuscular disease context, addressing the inclusion, diversity, equity, and accessibility needs of those with neuromuscular diseases, and building teamwork skills. Early evaluation of the first two modules indicates high satisfaction and knowledge gain. Through this process, we have learned about the barriers of patient-oriented research, how to support the required system culture shift, and how to plan for long-term sustainability.

Keywords

patient engagement, patient-oriented research, neuromuscular disease, online education, capacity building

Received: 10 March 2025; accepted: 18 November 2025

Highlights

- Patient-engagement is important to build relevant and purposeful research knowledge, especially in rare and neuromuscular diseases
- Capacity building is needed for the whole team, including researchers, clinicians and patient-partners to create meaningful engagement
- We present the co-development of an online, neuromuscular-specific patient engagement training
- Uptake of patient engagement is enhanced by considering sustainability for training modules, providing funding to support consequential engagement at the start of projects, and requiring patient-partnerships in grant competitions

Introduction

Over the past decade, the participation of individuals with lived experience in research has undergone significant evolution. There is now a strong emphasis on patient-oriented research (POR), a collaborative approach that actively engages patients, family members, and caregivers as integral partners throughout the research process.

[1]Neuromuscular Disease Network for Canada, Children's Hospital of Eastern Ontario Research Institute, Ottawa, Canada
[2]Department of Occupational Science & Occupational Therapy, University of British Columbia, Vancouver, Canada
[3]Department of Occupational Therapy, British Columbia Children's Hospital, Vancouver, Canada
[4]Muscular Dystrophy Canada, Toronto, Canada
[5]Division of Neurology, British Columbia Children's Hospital, Vancouver, Canada
[6]Faculty of Medicine, Department of Pediatrics, University of British Columbia, Vancouver, Canada

Corresponding author:
Patricia Mortenson, BC Children's Hospital, Occupational Therapy Department, K3-130–4480 Oak Street, Vancouver BC, Canada, V6H 3V4.
Email: patricia.mortenson@ubc.ca

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Essentially, POR means conducting research *with* patients rather than *for* patients,[1] thus ensuring that health research stays relevant and focused on improving outcomes that matter most to patients.[2]

Involving patient-partners is increasingly recognized as essential for high-quality health research. Benefits of this approach are highlighted in a 2019 review of studies conducted through the Patient-Centered Outcomes Research Institute (PCORI), which found an increased uptake in patient engagement, contributing to research that is more aligned with the needs of patients and clinicians.[3] The integration of patient-partners into health research lies at the convergence of two key ideas: 1) the concept that "research is care", and 2) the "nothing about us without us" principle widely embraced by the Disability Rights Movement.[4–6] This approach emphasizes the importance of including individuals with lived experience in the decision-making processes of research, ensuring that their perspectives are integral to shaping health outcomes and policies.[7] This represents a paradigm shift away from traditional research models, which treat patients as passive subjects, and instead emphasizes the inclusion of lived experiences and perspectives as essential to the research process. Unlike the term "patient," which typically refers to an individual receiving medical care, the term "patient-partner," describes someone with lived experience of a particular disease, either as a person directly affected, a family member or as a caregiver. A patient-partner collaborates and/or participates in decision-making roles within various health research contexts, from serving on grant review panels, advisory councils, to participating as an embedded member of a research team. This may involve prioritizing research ideas, and/or participating in study development, measurement selection, participant recruitment, data analysis, and dissemination of findings.[8–10]

Numerous components are needed to ensure meaningful patient-partner contributions throughout the research process. Careful planning is essential to maximize benefits, reduce risks, and ensure success.[5] Crucial elements include establishing trust and respect and fostering genuine relationships amongst all team members.[11–14] Additional considerations range from practical aspects, such as scheduling team meetings,[15] to issues of justice, such as ensuring diverse voices are represented,[13] and philosophical questions about the nature and ownership of knowledge.[12] Importantly, for POR to be effective, patients and researchers need to develop the necessary skills and competencies for this type of partnership to work.[11]

Capacity building needs to be considered and supported for effective patient engagement in research.[9] Training is recommended to cultivate successful collaboration, recognizing the diversity of experiences and needs within the research team.[8,9] Specifically, training should strive to build a "collective competence" in POR, nurture relationships and team dynamics, and foster a culture of co-learning to enhance team readiness.[14] Researchers require skills to support patient-engagement and participatory approaches, group processes, and effective communication.[11,16,17] Training programs for patient-partners should emphasize how to leverage lived experience in planning projects and disseminating findings, while deemphasizing the need to acquire specialized research skills.[11]

There is a growing recognition and application of POR in the realm of rare diseases and neuromuscular diseases (NMD).[18–20] Considering the broad spectrum of over 200 NMDs,[21] each individual brings a unique set of experiences, barriers, and challenges of their rare disease journey. Many NMDs are multisystemic disorders, affecting not only physical abilities but also mental health, cognition, endocrine, respiratory, cardiac, and bone health. As many are genetically inherited, multiple generations and other family members may also be affected by the same condition. This complexity can lead to immense economic burdens in affording treatments, medications, mobility equipment, and personal support care, resulting in social isolation.[22–24] Engaging with patient-partners with NMDs is paramount to understanding this complexity. Well-intentioned researchers may not consider the day-to-day realities of those living with NMD, and this in turn may result in less meaningful research, or research procedures that are difficult to access and engage in. Those affected, along with their families and caregivers, have unique experiences which can guide research questions and clinical practice to maximally benefit patient outcomes.[25] Patient-partners provide depth and context to rare disease research, increase innovation, facilitate improved recruitment and research procedures, improve study completion rates, enhance the relevance, utility and application of research, and aid in the dissemination of information to medical and NMD communities.[326–28] However, despite the importance of patient engagement, few NMD research studies to date use POR methods.

Numerous POR training and capacity building supports exist for other populations and conditions.[2,8,29,30] However, there are none that are specifically tailored with an NMD focus and designed to be accessible for and considerate of the unique challenges of living with an NMD. To effectively conduct POR within this population, it is imperative to develop capacity-building training that acknowledges the experiences of individuals with NMDs. This includes addressing the wide range of potential NMD related participation barriers, such as fatigue (almost all NMDs), cognitive and language understanding (e.g., myotonic dystrophy), dysarthria and respiratory illness vulnerability (e.g., spinal muscular atrophy), physical accessibility and equipment needs of many NMDs, and related caregiver assistance barriers (scheduling limitations and availability). All these challenges can limit attendance at research team meetings, reading and understanding of communications, ability to provide feedback, or attendance and participation in activities. The purpose of this project was to develop a set of online, self-directed training modules applicable to NMD research;

from which patient and family partners, researchers, trainees, and others on research teams can learn about researcher-patient partnerships and discover ways to conduct research that is accessible and meaningful to individuals living with NMDs.

Methods

A dedicated multi-stakeholder team was formed to determine the learning objectives, training content and user interface. The 'Expert Patient Capacity Building (EPCB) team' consisted of four patient-partners (CK, CM, LN, MT), representatives from a national patient organization, Muscular Dystrophy Canada (MDC) (HO), the Neuromuscular Disease Network for Canada (NMD4C) (a pan-Canadian network connecting clinical, scientific, technical and patient experience to enhance care and research in NMD) (EB), one researcher, one clinician (KS), and one project coordinator/clinician (PM). POR principles and practices were integrated into all phases of the current project. Patient-partners on the team each had a different NMD condition with different related experiences and challenges. The full project occurred in two major phases and developed three unique modules (see Figure 1 for project flow chart and Supplemental Figure 1 for patient-partner visualization of the project). Patient-partners were involved in each phase and activity, including the identification of search terms for a landscape analysis, collection and analysis of data (i.e., resources), design of the capacity building online training modules, and implementation and dissemination of all the modules.

Phase one: landscape analysis

The goal of Phase 1 was to complete a landscape analysis of the available POR resources and best practices and to retrieve, screen, and categorize the evidence collected. The outcomes of the landscape analysis were: (a) to select relevant resources to be included in an online resources repository for POR in NMD research, and (b) to identify gaps in the existing resources that could guide the development of a new online POR capacity building training for NMD research. Search terms included: rare disease, neuromuscular diseases, capacity building, training, patient engagement, patient partner, patient participation, and/or patient-oriented research. Searched databases included: Google, Google scholar, OVID Medline, and EBSCO CINAHL, all with English language limits. The grey literature search was iterative to identify tools and resources for POR captured in workshops, conferences, websites, repositories, and other resource materials. Websites of known POR organizations (e.g., Canadian Institutes of Health Research Strategy for Patient-Oriented Research, Patient-Centered Outcomes Research Institute) were also scanned for linked resources. One author (PM) scanned, downloaded and categorized the published article citations onto a Microsoft Word document; online sources were collected and categorized on an Excel database, with two authors (CK, HO) confirming the resources and categories. This was then converted into an online resource repository on the NMD4C website.

The EPCB team discussed gaps in the existing resources in relation to rare disease and NMD research. Three team members (CK, HO, PM) used an inductive approach[31] to identify major gaps that emerged from the landscape analysis. These identified gaps were then used to inform the development of the POR capacity building training modules for patient-partners and researchers involved in NMD research (Phase 2). To build our collective skills and identify gaps in trainings, team members (CK, CM, PM, HO, MT) completed and evaluated engagement trainings available at the time, including the Patient-Oriented Research Curriculum in Child health modules[29] and the Maritime SPOR SUPPORT Unit Patient-Oriented Research Training Online Practicum.[32] Midway through our project, the Canadian Institutes of Health Research - Institute of Musculoskeletal Health and Arthritis (CIHR-IMHA) launched POR training modules which our team then reviewed.

Phase two: POR in NMD research online capacity building training development

We followed a 'backward design process' to develop the framework for the capacity building training.[33] Backward design begins with identifying learning needs and desired actions/skills, followed by specific learning objectives and competencies (the evidence of learning), and ends with planning learning activities to support the learning objectives. We developed our training, 'Patient-Oriented Research Training in Neuromuscular Disease (imPORTND)'[34] in four steps; we: (a) established the key messages and skills, (b) wrote the matching learning objectives, (c) created the learning activities, and (d) built the online platform. Adult learning principles, including offering learning choices, grounding in real life context and application, and incorporating social learning opportunities informed the development of the learning activities.[35] The EPCB team collaborated through online gatherings to discuss results of the landscape analysis, and the development, dissemination, evaluation and sustainability of the training modules. To facilitate sustainability, the evaluation process utilized the RE-AIM Framework,[36] which examines aspects of reach, effectiveness, adoption, implementation, and maintenance.

Phase three: launching the modules

As we built the modules, we embedded methods for the project launch, including knowledge translation activities, dissemination, and evaluation. This involved strategies to build awareness and interest in patient engagement prior to the launch and dissemination approaches once the modules

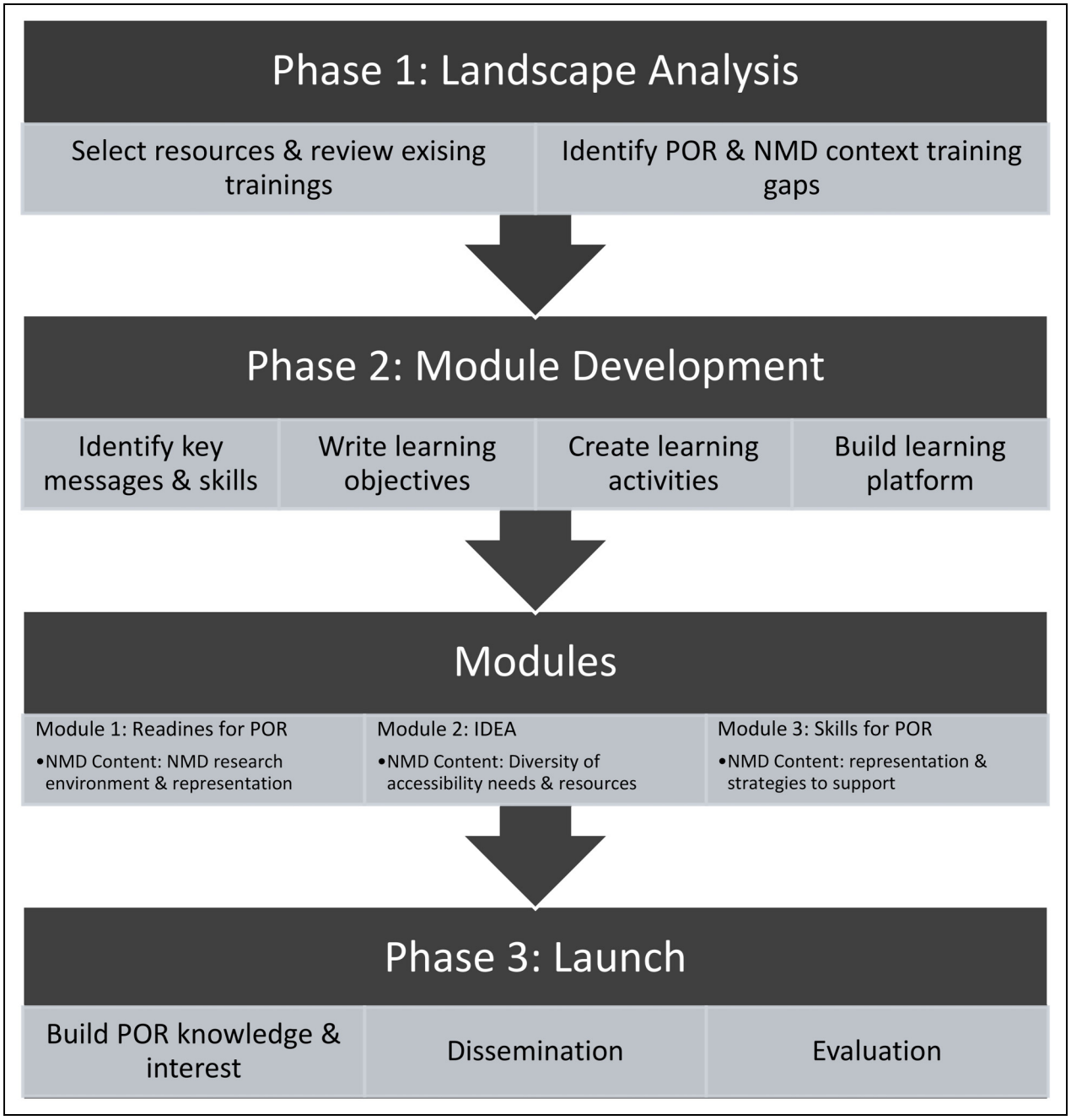


Figure 1. Project flow chart. Abbreviations. IDEA, Inclusion, diversity, equity, accessibility; NMD, neuromuscular disease; POR, patient-oriented research.

were ready. Finally, we tracked learner participation, completion and evaluations using features of the Moodle platform.

Results

Phase one: landscape analysis

A total of 80 resources were included in the online repository (Table 1), sorted into nine major categories. The online repository serves as a library of mostly Canadian resources for stakeholders engaged in POR, including people affected by NMDs, researchers, and advocates. The repository is housed on the NMD4C website and is maintained by the NMD4C communications coordinator, together with MDC.[37]

By consensus, we identified these gaps in the current trainings and resources: 1) NMD context and environment; 2) focus on inclusion, diversity, equity and accessibility needs (accessibility being particularly important to an

Table 1. Categories and resources for online resource repository.

Resource Category	Resources Included in Online Repository n=
Introduction and overview of POR	5
Online POR training	13
Patient engagement	20
Communication and writing for patient-partners	4
POR project planning	5
Patient-partner compensation	4
Equity, diversity and inclusion (EDI)	11
POR project evaluation	7
POR project reporting	1

NMD context); 3) consideration of motivations; 4) skills for building and working in, teams; 5) support for patients and researchers to exchange unexpected knowledge through patients sharing impactful stories and researchers listening; 6) considerations for engagement planning; and lastly7) representation and visibility, as existing POR training lacked examples featuring patient-partners with a range of disabilities, as are experienced with NMD.

The first gap specifically addressed the need to describe the complex NMD research environment. With over 200 rare conditions (some extremely rare), many different organizations and institutions involved in NMD research, creating a layered and confusing research environment. The second gap was also unique to patient engagement in the NMD context. Given the different health and disability complexities represented, supporting patient-partners to participate in research requires considering and addressing a wide range of different physical, health, and communication barriers. This extends to the importance of terminology and language-use and reflecting on unconscious biases and stigmas. The remaining gaps, while not necessarily unique to NMD, were not addressed by other resources. However, two principles made these gaps important to address, first, the need to consolidate training to make it accessible and doable, and second, the importance of representation. In a population that has had so little patient engagement in research to date and for whom many face disability discrimination, seeing oneself in a role and others appreciating the potential is critical.[38]

Phase two: online capacity building training development

Step one: development of the learning objectives. The EPCB team determined the desired learning messages and key skills through discussion and consensus and incorporating the identified gaps. These were divided into three main themes that became one welcome module and three content modules (introduction to POR in NMD context; inclusion, diversity, equity, and accessibility (IDEA); and skills for POR) (see Figure 2). Key skills were developed into 22 learning objectives and competencies, some directed primarily for patient-partners, others for researchers/research teams, and some applicable to all, with the thought that certain skills, like team building, are best learned together (see Supplementary Tables 1, 2, 3). To limit duplication, the imPORTND modules were developed to complement the newly published CIHR-IMHA modules and to link to resources found through the landscaping phase.

Step two: module content development. During the platform design and development of learning activities to meet the learning objectives, the team aimed to create content that was inclusive by design and responsive to the diversity of NMD accessibility needs. First, the content followed the Web Content Accessibility Guidelines 2.1,[39] an internationally recognized set of recommendations for improved web accessibility. This ensures that content is accessible to people with a variety of vision, hearing, mobility and cognitive needs. This included creating transcripts or closed captioning options for videos and adjusting font and colour usage. Second, plain language was used wherever possible to eliminate barriers to understanding, ensure optimal uptake, and to empower patient-partners. Third, representations of people living with NMD were used in examples, case scenarios and module knowledge tests. And fourth, it was determined that online training should track learning and completion of each module. Finally, we translated the full training, including video voice overs, into French to provide the training in both national languages (RE-AIM outcome: Reach).

In building our modules, we chose the Moodle platform, a free and open-source learning management system (https://moodle.org/), to host the training. Moodle's features, such as content pages, discussion boards, ability to link in external videos and content, quiz features, and ability to track participation and completion, met our need for a diversity of learning activities. Throughout content building, we used a shared Word document to track content ideas and appropriate resources, each matched to specific learning objectives, reflecting the backward-design process (see Supplementary Tables 1, 2, 3). Text, video scripts, visuals, and graphics were developed and reviewed by the team. This iterative process made best use of our different perspectives (patient, researcher, clinician) and allowed for our team to learn from each other and adjust the content to best meet the needs of mixed users. In addition, following adult learning principals, we built the modules with elements of choice and the ability to quickly revisit specific content and resources to provide 'just-in-time' follow-up learning opportunities. Finally, a small research group, engaging patient-partners in the creation of a core data set for Duchenne muscular dystrophy, pilot tested the first two imPORTND modules (RE-AIM outcome: Effectiveness).[40] Final adjustments were made based on feedback from this group.

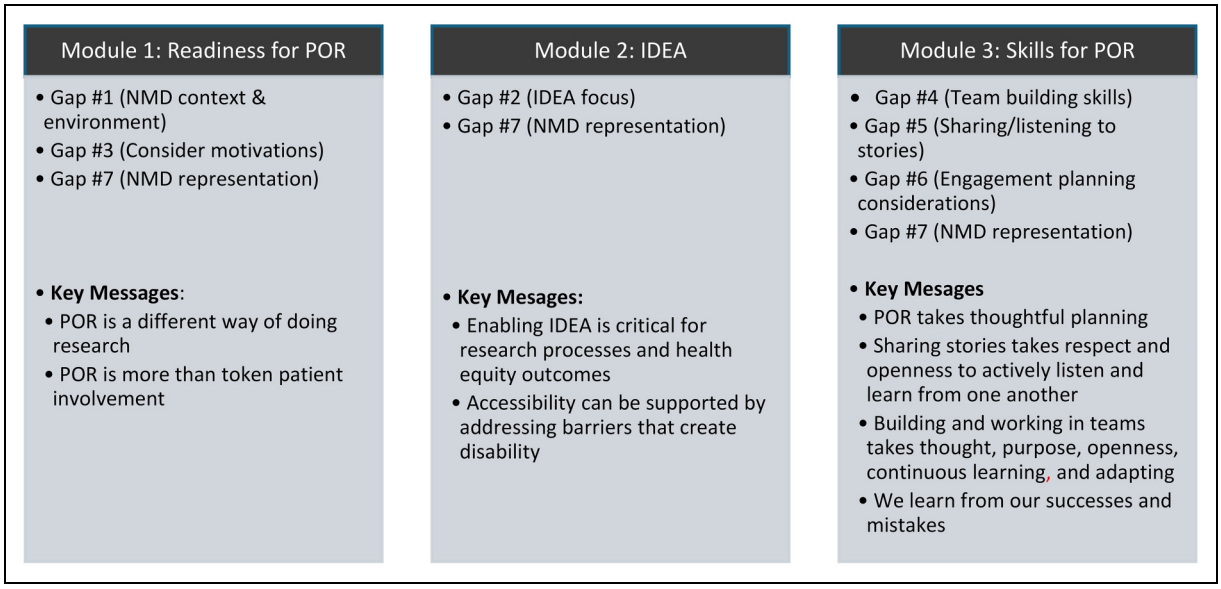


Figure 2. Identified gaps and key messages module themes. Abbreviations. IDEA, inclusion, diversity, equity and accessibility; NMD, neuromuscular disease; POR, patient-oriented research.

ImPORTND training content and learning activities

Module 1: readiness for patient-oriented research. The first imPORTND module (see Supplementary Table 1) defines POR within a NMD context, including how lived experience can enhance NMD research. The complex NMD environment is introduced, including the key organizations that partner in health research, such as the numerous patient and research organizations, the Canadian NMD network and the Canadian NMD registry, hospitals and clinics, and universities. The different roles and responsibilities of research team members within these organizations is further explained. This module also explores the different motivations people may have in participating and engaging in research. The underlying message is that POR involves mutual trust and constant relationship building, and tips and resources are featured for how to effectively do this work. This includes a video example of a NMD researcher and a patient-partner with NMD related dysarthria in conversation about relationships in research, modelling communication and visibility. Researchers are provided practical tools to support patient-partners, including information on compensation, reimbursement and how to acknowledge and report the impact of POR in reports and papers. The module concludes with contact information for potential patient-partners and researchers to use to facilitate connections and coordination by the patient organization, MDC (RE-AIM outcome: Adoption).

Module 2: inclusion/diversity/equity/accessibility (IDEA). Module 2 (Supplementary Table 2) presents learning to equip research teams and patient-partners with the skills and resources needed to support inclusive, diverse, equitable and accessible (IDEA) research. Rather than addressing NMD related research participation barriers by each NMD diagnosis, the team made two decisions. First, to equip users more broadly through the lens of IDEA and the social model of disability,[7] and second, to have all users complete this learning. Not all researchers and trainees understand the diversity of NMD needs, and those with lived experience of one NMD condition may not understand that of another. To start, learners are introduced to IDEA and complete an activity to prompt reflection on their biases by completing an online bias test. The module then links to teaching content about the social model of disability as a lens to consider all types of accessibility barriers. As many people with NMDs have mobility needs, learners are introduced to different types of mobility devices, and content is linked and provided to discuss how to communicate and share information with those with learning and communication needs. For researchers specifically, resources are provided to help describe the research cycle process in a plain language, accessible manner for those supporting participation of patient-partners with cognitive support needs, and an accessibility checklist of research environments and processes is available for download.[41]

Module 3: skills for patient-oriented research. Module 3 (Supplementary Table 3) focuses on specific skills to help with POR. This includes how to develop an effective patient engagement plan, how to share and listen to patient stories, how to work effectively in groups, and how to evaluate the project engagement. Module 3 also

Table 2. Completed modules.

	Completed Module 1 n=	Completed Module 2 n=
Patient-partner	22	18
Clinician researcher	6	4
Senior researcher/principal investigator	4	4
Trainee/early career researcher	8	5
Research coordinator/manager	4	4
Other	3	3
Total	47	38

provides practical information about the stages of team development along with identifying the needs for each stage and group activities to foster relationships and trust. This module also links to the CIHR-IMHA trainings. To address NMD visibility and representation in research processes, Module 3 includes a case study activity that asks participants to read a vignette about a person diagnosed with Charcot-Marie-Tooth disease and identify what matters most to them, along with the accommodations or considerations needed if they joined a research team.

Phase three: launching the modules

To build knowledge and interest for POR, the EPCB team delivered a network webinar, and presented the project 'in progress' at two national network meetings (RE-AIM outcome: Reach). Dissemination included email notifications to all individuals registered within MDC and NMD4C and advertising in e-blasts and newsletters using consistent graphics and messaging (RE-AIM outcome: Adoption).

To date, 47 people have completed Module 1, and 38 have completed Module 2, with interested patient-partners the most involved, followed by trainees and early career researchers (see Table 2). Satisfaction with the training has been highly rated to date. For Module 1, 44 of 47 people (94%) agreed or strongly agreed that they knew more about POR following the module, and for 37 (79%), learning this from an NMD perspective and context, as presented in the module, was important (see Figure 3). For 42 people (89%), Module 1 took an appropriate length of time to complete and 44 (94%) would revisit the site to access the resources. For Module 2, 29 of 38 people (76%) agreed or strongly agreed that they learned more about IDEA principles through the module, and 36 (95%) reported that learning this through an NMD lens, as presented, was important (see Figure 4). The two that were either neutral or disagreed on this point were patient-partners, who may have gained this knowledge already through lived experience. For 33 people (87%), Module 2 took an appropriate length of time to complete, and 28 (74%) would revisit it in the future to access the resources.

Evaluation included tracking user perceptions of the intent and meaning of the modules, and their 'take home messages'. For patients, the learnings demonstrated a shift

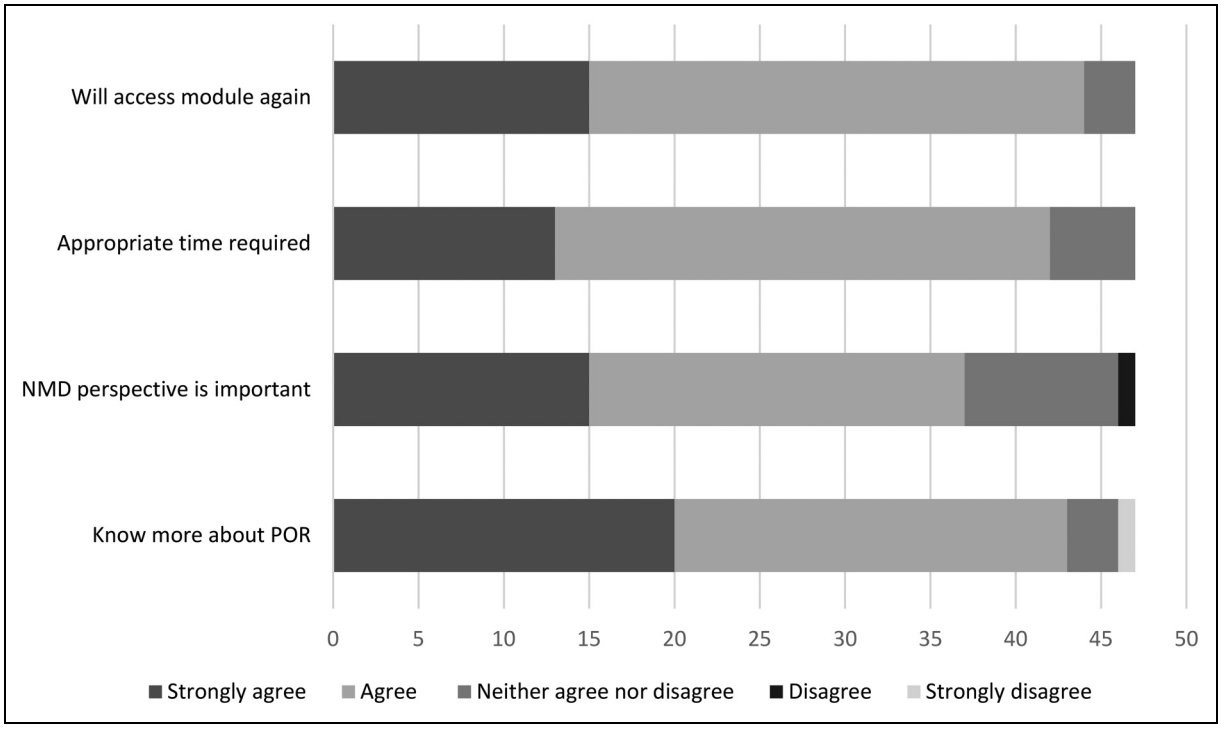


Figure 3. Module 1 program evaluation data.

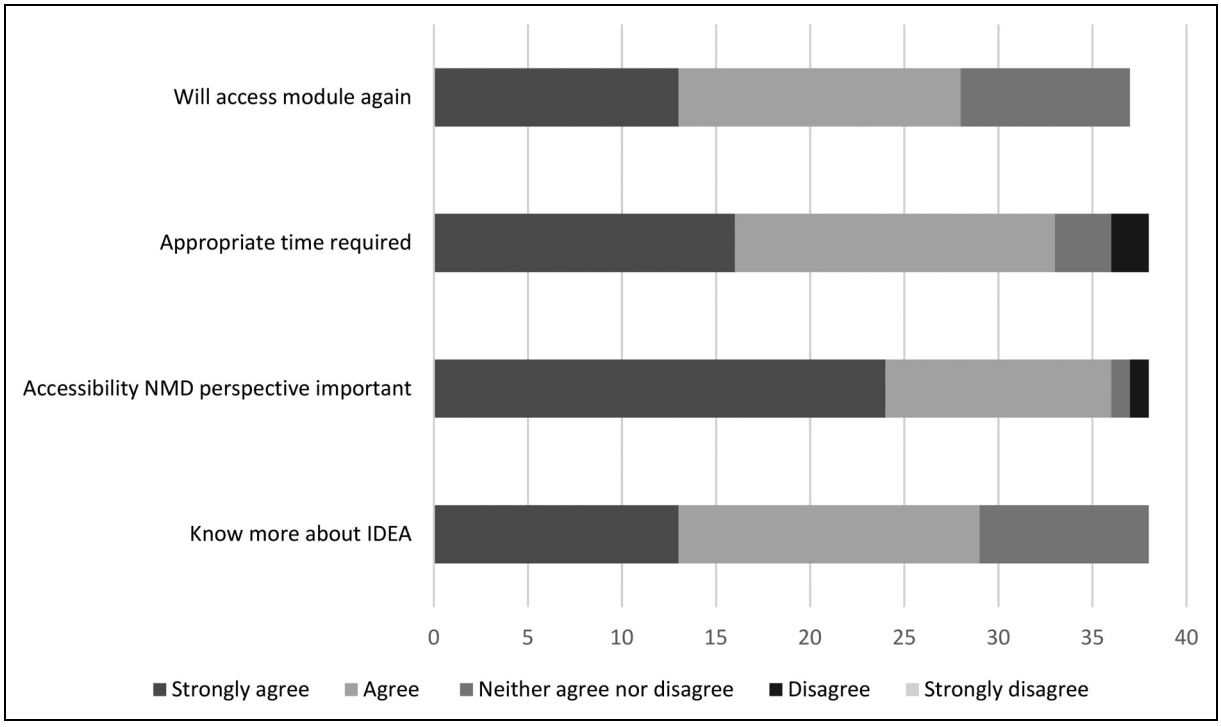


Figure 4. Module 2 program evaluation data.

in research approach and opportunities. From one patient, "as a person with a neuromuscular disease, I am very happy learning how this subject is being taught, and that energy is going into being more understanding of us". Another noted, "having a healthy relationship and communication between researchers and patients can lead to better outcomes for the research being done". For researchers, comments focused on how including patient-partners can change research processes and outcomes. A senior researcher wrote, "we can learn so much more about research priority from the patients", and a trainee/early career researcher noted to, "consider IDEA at the onset of research redesign, consider communication strategies to address any unique needs of your patient partners and community". The few negative comments related to practicalities of online learning and internet connectivity, such as buffering speed to view videos. Online learning can present challenges, but one participant noted, "my attention span for online learning has decreased in the last few years but held out for this complete module".

Discussion

Our imPORTND project addresses notable voids in patient-engagement knowledge and available resources. Initially we confirmed the lack of POR resources and optimal methodologies applicable to NMDs. We then addressed this gap by creating the first POR training modules unique to the needs of NMD patients and their research and clinical care teams. Through this work our EPCB team gained a deeper understanding of the barriers to this type of work, the research culture shift required, and how to build for sustainability.

Barriers to POR

We designed our imPORTND modules with the intention that patient-partners, and research and clinical teams build relationships and learn together through content that is reflective of all perspectives. However, there can be barriers to POR participation for both patients and professionals. Accessibility can be a challenge for patients, impacting the diversity and representation of patient-partner recruits.[41,42] In addition, the complexities of living with a rare condition can limit patient involvement even further,[1] and for those living with progressive neuromuscular conditions, the impacts extend to family and caregivers. Patients and caregivers can spend considerable time advocating for research, funding, financial support, and access to treatments, leaving little time for research involvement. These sentiments are echoed in patient engagement in other rare disease research.[1,16] The co-design of imPORTND mitigates some of these barriers and facilitates POR as it was developed in collaboration with patient-partners and NMD-organizations, which have diverse reach and can thus facilitate dissemination of the training program and uptake among patient-partner groups. For example, imPORTND has an entire module dedicated to supporting

accessibility for patient-partners in research activities. Finally, imPORTND is self-paced and has flexible learning content, allowing all to move through modules on their own time and capacity.

Ensuring diversity of patient voices within research is important to expanding reach and applicability of research but is difficult to achieve.[43,44] A survey examining the demographics of patient-partners in multiple Canadian jurisdictions and health organizations found that patient-partners are predominantly female, university-educated, white, and have a household income greater than $90,000 a year.[42] In the case of rare diseases, with small potential patient-partner pools, the same people tend to be repeatedly invited if they demonstrate interest and aptitude. In addition, founder effects seen in some NMDs create concentrated, regional distributions of specific conditions, sometimes in rural locations far from research 'hubs'.[45,46] This highlights the need to target patient engagement at regional and community levels to ensure representation of these rare conditions. Failing to include representative patient-partners can have a direct impact on the identification of research priorities and knowledge translation initiatives to reach the larger population.[16] imPORTND was developed to build the capacity and inclusion of more patient-partners so researchers are not continually involving the same patient-partners.

Barriers for researchers engaging with patient-partners include poor understanding of the purposes, meaning, and methodology of POR. Both veteran and early career researchers report having only basic knowledge of POR and only one third of researchers perceive POR as actively involving patient-partners in all phases of research.[1,47] These findings imply that researchers may be unclear of the potential roles for patient-partners. In particular, patient-partner engagement in bench science remains difficult.[48] While grant competitions increasingly require patient-partner participation, few do so for basic science research. This type of research is vital for making advances in the treatment of genetically based or autoimmune NMDs. Potential patient-partner contributions in basic science research include informing priorities and proposals, developing effective recruitment strategies when needed, and/or participating in 'lay' knowledge translation messaging.

Partnering scientists early in their career with patient-partners may be a key strategy to increasing uptake and developing inclusive methods while infusing laboratory research with meaning. At a 2024 NMD4C basic science summer school, NMD research trainees met with patients to hear their stories. This experience helped trainees connect the meaning of their laboratory research and its impact on those living with an NMD. This resulted in a trainee-driven push for further engagement, including their completion of the imPORTND training modules, and the development of a 'Building Bridges" learning series. This series focuses on collaboration between patient partners and researchers to ensure that scientific communication is accessible, empowering, and inclusive.

While training can increase knowledge and skills to improve POR uptake, it doesn't address systemic barriers in research processes, funding and connecting with patients. While our imPORTND modules highlight the importance of early involvement, our team recognized a significant barrier to this ideal. Best practice includes compensating patient-partners for their time and reimbursing them for any costs incurred for their participation (e.g., caregiver support required, parking, childcare, etc.),[49] however projects rarely have funding available at these early stages, and not all developed proposals receive funding. For a true commitment to patient engagement, research funding models need to change to support patient-partners from the start. Until this happens, our partnering patient organization, MDC, has made funds available for researchers to compensate patient-partners for their time involvement in the initial phases of project development and grant application. This is supported by imPORTND, which incorporates a process to build a bank of trained patient-partners to be prospectively matched by our partnering patient organization with researchers early in grant development.

Shifting research culture

During our work on this project, we became increasingly aware of the culture shift required for integration of POR. When our EPCB team began this project, it was one of seven separate project streams within the NMD4C. In a systematic review of competencies required by researchers, development of community engagement skills was viewed as a low priority, with only one of 16 reports identifying this as a required research skill.[50] However, by involving patient-partners as co-presenters and co-developers in our knowledge translation activities, including a webinar, and at network meetings, the perceived importance of patient-engagement within the network evolved. Building capacity for patient engagement is no longer a separate project in NMD4C but is now a cross-cutting theme, and patient-partners are 'embedded' members in all the network's current work. As noted, however, the uptake of our modules has been higher with patients than with research teams, highlighting the need for future projects to focus on generating continuous buy-in amongst researchers.

Directing the utilization of research funds is another tangible mechanism to demonstrate commitment and a shift in practice. To further promote this behaviour our supporting patient organization, MDC, has made patient engagement a criterion for submitted applications in their research grant competitions. This has increased patient-partnered work by 50%. Additionally, patient-partners are involved as reviewers in the grant review process. Involvement at this level gives patient voice and

representation in directing research funding and prioritization but also helps provide critical feedback to investigators when a project's ranking suffers for not including patient-partners.

While capacity building is just one piece of POR it is only through authentic partnerships between patients and researchers that research can advance for rare diseases. Fagan et al.[51] suggest that research institutions utilize existing structures, like patient organizations (e.g., Muscular Dystrophy Canada), to facilitate POR. imPORTND actively focuses on establishing and maintaining relationships through training and is a catalyst for matching patient-partners with appropriate research studies.

Future

The need for continuous maintenance and sustainability is a concern with online training and resources. We developed imPORTND with sustainability at the forefront to ensure that the training remains live, updated and continuously relevant. To ensure maintenance, quarterly reviews of the content take place to ensure that links and videos are operational. Survey evaluation is built into the end of each module, to track 'roles' of who completes the training and to seek feedback for learner experience, and suggestions for improvement and relevance. Future projects and initiatives should focus on ensuring researchers understand the value of POR and tracking practice shifts, including the diversity and numbers of patient-partners involved, the numbers of network associated projects that include patient-partners, and in what type of research, and how the patient community perceives the relevance of research outputs. We anticipate that the training may need updating every three years and have built in mechanisms to inform the next iteration.

Conclusion

The aim of this project was to develop a capacity-building training program focused on POR specifically tailored for researchers and patient-partners involved in NMD research. We achieved this goal but also discovered that the process was as important as the product. Engagement with patient-partners throughout the project helped the team consider the barriers and research culture from different perspectives. This enabled us to address some of these barriers directly in the design and content of the training. It also helped establish better processes for linking patients with researchers, and in working with our patient organization, we were able to address some of the financial and structural funding barriers to POR. Most importantly, it helped us communicate the importance of POR more effectively to the entire network and establish 'buy-in' from researchers and patients. This has extended to having patient-partners further embedded within the network and established respectful and beneficial connections. At a time when research is shifting towards treatment possibilities for rare neuromuscular diseases, having a fully engaged community is critical. Our training project has started to shift how that research activity and associated networking is being co-constructed in Canada. There is still much work to do, but this has been a meaningful start.

Statements and declarations

Dr K. Selby has been principal investigator in clinical trials with Biogen, Roche, Italfarmico and Reverogen. She received an honorarium for consultant advisory committee work for Roche and Novartis and received an honorarium for a presentation from Novartis. Dr Selby decares that she has no declarations of interest regarding this manuscript.

Acknowledgements

The authors acknowledge James Davis, Communications and Administrative Coordinator for the Neuromuscular Disease Network for Canada, for his technology assistance and support in uploading our resource repository and developing the learning platform courses and uploading and managing learning materials. We also acknowledge Dr Cynthia Gagnon's contributions to the conceptualization, funding acquisition and methodology of the project and her French language video content.

Ethical considerations

Research ethics was not needed for this education project. Data collected was in keeping with quality improvement and course evaluation.

Author contributions

Patricia Mortenson: Conceptualization, Methodology, Formal Analysis, Investigation, Resources, Writing - original draft preparation, review and editing, Visualization, Project administration.
Homira Osman: Conceptualization, Methodology, Formal Analysis, Investigation, Resources, Writing - original draft preparation, review and editing, Supervision, Funding acquisition.
Erin Beattie: Conceptualization, Methodology, Writing - review and editing, Project administration, Supervision, Funding acquisition.
Corinne Kagan: Conceptualization, Methodology, Resources, Writing - review and editing.
Victoria Larocca: Writing - original draft preparation, review and editing
Claudia Maltais: Conceptualization, Methodology, Resources, Writing - review and editing.
Linda Niksic: Conceptualization, Methodology, Resources, Writing - review and editing.
Margo Thompson: Conceptualization, Methodology, Resources, Writing - review and editing.
Kathryn Selby: Conceptualization, Methodology, Writing - review and editing, Project administration, Supervision, Funding acquisition.

Funding

This project was supported by the Neuromuscular Disease Network for Canada (NMD4C) via Canadian Institutes of Health Research and Muscular Dystrophy Canada grants (Network Grant: OR2-189333; Network Catalyst Grant: NG2-170044); Patricia Mortenson is funded by a University of British Columbia Four Year Doctoral Fellowship.
Institute of Musculoskeletal Health and Arthritis, Muscular Dystrophy Canada, (grant number OR2-189333, Network Catalyst Grant: NG2-170044).

Declaration of conflicting interests

The authors declared no potential conflicts of interest with respect to the research, authorship, and/or publication of this article.

Data availability

All planning details for the project are available in the supplementary tables. The online training is freely available at https://importnd.neuromuscularnetwork.ca/.

Supplemental material

Supplemental material for this article is available online.

ORCID iDs

Patricia Mortenson https://orcid.org/0000-0001-6071-4337
Homira Osman https://orcid.org/0009-0001-4225-9075
Erin Beattie https://orcid.org/0009-0004-5392-7915
Kathryn Selby https://orcid.org/0000-0001-9239-1704

References

1. Velvin G, Hartman T and Bathen T. Patient involvement in rare diseases research: a scoping review of the literature and mixed method evaluation of Norwegian researchers' experiences and perceptions. *Orphanet J Rare Dis* 2022; 17: 12.
2. Getchell L, Bernstein E, Fowler E, et al. Program report: kidneyPRO, a web-based training module for patient engagement in kidney research. *Can J Kidney Health Dis* 2020; 7. https://doi.org/10.1177/2054358120979255
3. Forsythe LP, Carman KL, Szydlowski V, et al. Patient engagement in research: early findings from the patient-centered outcomes research institute. *Health Aff* 2019; 38: 359–367.
4. Dember LM. What patients teach us about patient engagement in research. *Clin J Am Soc Nephrol* 2022; 17: 176–178.
5. Manafo E, Petermann L, Mason-Lai P, et al. Patient engagement in Canada: a scoping review of the 'how'and 'what'of patient engagement in health research. *Health Res Policy Syst* 2018; 16: 1–11.
6. King N. Research is care. *Hastings Cent Rep* 2012; 42: 7.
7. Oliver M. *Understanding disability: from theory to practice*. 2nd ed. London: Bloomsbury publishing, 2018.
8. Bell T, Vat LE, McGavin C, et al. Co-building a patient-oriented research curriculum in Canada. *Res Involv Engagem* 2019; 5: 1–13.
9. Canadian Institutes of Health Research (CIHR). Strategy for patient-oriented research: patient engagement framework, https://www.cihr-irsc.gc.ca/e/41204.html (2015, accessed 23 February 2025).
10. Demian MN, Lam NN, Mac-Way F, et al. Opportunities for engaging patients in kidney research. *Can J Kidney Health Dis* 2017; 4. https://doi.org/10.1177/2054358117703070
11. Frisch N, Atherton P, Doyle-Waters MM, et al. Patient-oriented research competencies in health (PORCH) for researchers, patients, healthcare providers, and decision-makers: results of a scoping review. *Res Involv Engagem* 2020; 6: 1–14.
12. Leese J, Macdonald G, Kerr S, et al. 'Adding another spinning plate to an already busy life'. Benefits and risks in patient partner–researcher relationships: a qualitative study of patient partners' experiences in a Canadian health research setting. *BMJ Open* 2018; 8: e022154.
13. Pozniak K, Buchanan F, Cross A, et al. Building a culture of engagement at a research centre for childhood disability. *Res Involv Engagem* 2021; 7: 1–15.
14. Zibrowski E, Carr T, McDonald S, et al. A rapid realist review of patient engagement in patient-oriented research and health care system impacts: part one. *Res Involv Engagem* 2021; 7: 1–13.
15. Hamilton CB, Hoens AM, Backman CL, et al. An empirically based conceptual framework for fostering meaningful patient engagement in research. *Health Expect* 2018; 21: 396–406.
16. Bishop AC, Elliott MJ and Cassidy C. Moving patient-oriented research forward: thoughts from the next generation of knowledge translation researchers. *Res Involv Engagem* 2018; 4: 1–6.
17. Chudyk AM, Stoddard R, Duhamel TA, et al. Future directions for patient engagement in research: a participatory workshop with Canadian patient partners and academic researchers. *Health Res Policy Syst* 2024; 22: 24.
18. Ambrosini A, Quinlivan R, Sansone VA, et al. "Be an ambassador for change that you would like to see": a call to action to all stakeholders for co-creation in healthcare and medical research to improve quality of life of people with a neuromuscular disease. *Orphanet J Rare Dis* 2019; 14: 1–12.
19. Furlong P, Dugar A and White M. Patient engagement in clinical trial design for rare neuromuscular disorders: impact on the DELIVER and ACHIEVE clinical trials. *Res Involv Engagem* 2024; 10. https://doi.org/10.1186/s40900-023-00535-1
20. McCray AT, LeBlanc K and Network UD. Focus: rare disease: patients as partners in rare disease diagnosis and research. *Yale J Biol Med* 2021; 94: 687.
21. Ricci G, Torri F, Bianchi F, et al. Frailties and critical issues in neuromuscular diseases highlighted by SARS-CoV-2 pandemic: how many patients are still "invisible"? *Acta Myol* 2022; 41: 24–29.

22. Handberg C and Werlauff U. People with neuromuscular diseases and their relatives' perspectives on challenges in everyday life and healthcare. *Neurodegenerative Dis Manage* 2023; 13: 289–302.
23. Hughes T, Howard JF, Silvestri NJ, et al. The economic burden of individuals living with generalized myasthenia gravis and facing social determinants of health challenges. *Front Public Health* 2023; 11: 1247931.
24. Rodríguez AA, Martínez Ó, Amayra I, et al. Diseases costs and impact of the caring role on informal carers of children with neuromuscular disease. *Int J Environ Res Public Health* 2021; 18: 2991.
25. Dillon EC, Tuzzio L, Madrid S, et al. Measuring the impact of patient-engaged research: how a methods workshop identified critical outcomes of research engagement. *J Patient Cent Res Rev* 2017; 4: 37.
26. Badiu C, Bonomi M, Borshchevsky I, et al. Developing and evaluating rare disease educational materials co-created by expert clinicians and patients: the paradigm of congenital hypogonadotropic hypogonadism. *Orphanet J Rare Dis* 2017; 12: 1–9.
27. Hamakawa N, Kogetsu A, Isono M, et al. The practice of active patient involvement in rare disease research using ICT: experiences and lessons from the RUDY JAPAN project. *Res Involv Engagem* 2021; 7: 1–15.
28. Young K, Kaminstein D, Olivos A, et al. Patient involvement in medical research: what patients and physicians learn from each other. *Orphanet J Rare Dis* 2019; 14: 1–7.
29. Macarthur C, Walsh CM, Buchanan F, et al. Development of the patient-oriented research curriculum in child health (PORCCH). *Res Involv Engagem* 2021; 7: 1–5.
30. Richards DP, Twomey R, Flynn T, et al. Patient engagement in a Canadian health research funding institute: implementation and impact. *BMJ Open* 2024; 14: e082502.
31. Spencer L, Ritchie J and O'Connor W. Analysis: practices, principles and processes. In: J Ritchie and J Lewis (eds) *Qualitative research practice: a guide for social science students and researchers*. London: Sage, 2003, pp.199–218.
32. Maritime SPOR SUPPORT Unit. Patient-oriented research training online practicum, https://mssu.ca/get-involved/attend-training/ (last accessed 23 February 2025).
33. Wiggins G and McTighe J. *Understanding by design*. 2nd ed. Alexandria, VA: Association for Supervision and Curriculum Development, 2005.
34. Mortenson P, Kagan C, Maltais C, et al. imPORTND: patient-oriented research training in neuromuscular diseases, https://importnd.neuromuscularnetwork.ca/ (2023, last accessed 28 February 2025).
35. Ornelles C, Ray AB and Wells JC. Designing online courses in teacher education to enhance adult learner engagement. *Int J Teach Learn Higher Educ* 2019; 31: 547–557.
36. Glasgow RE, Vogt TM and Boles SM. Evaluating the public health impact of health promotion interventions: the RE-AIM framework. *Am J Public Health* 1999; 89: 1322–1327.
37. The Neuromuscular Disease Network for Canada. Patient oriented research resources, https://neuromuscularnetwork.ca/cross-cutting-network-themes/expert-patient-capacity-building/patient-oriented-research-resources (2023, last accessed 28 February 2025).
38. Rice C, Chandler E, Harrison E, et al. Project ReVision: disability at the edges of representation. *Disabil Soc* 2015; 30: 513–527.
39. Kirkpatrick A, Connor J and Cooper M. Web content accessibility guidelines (WCAG) 2.1, https://www.w3.org/TR/WCAG20/ (2018, accessed 22 February 2023).
40. Bourcier D, Koenig N, Hodgkinson V, et al. Mapping a national duchenne muscular dystrophy registry to the international classification of functioning, disability, and health. *Can J Neurol Sci* 2023; 50: S78–S78.
41. Browne J and Dorris ER. What can we learn from a human-rights based approach to disability for public and patient involvement in research? *Front Rehabil Sci* 2022; 3: 878231.
42. Abelson J, Canfield C, Leslie M, et al. Understanding patient partnership in health systems: lessons from the Canadian patient partner survey. *BMJ Open* 2022; 12: e061465.
43. Reynolds J, Ogden M and Beresford R. Conceptualising and constructing 'diversity' through experiences of public and patient involvement in health research. *Res Involv Engagem* 2021; 7: 1–11.
44. Shimmin C, Wittmeier KD, Lavoie JG, et al. Moving towards a more inclusive patient and public involvement in health research paradigm: the incorporation of a trauma-informed intersectional analysis. *BMC Health Serv Res* 2017; 17: 1–10.
45. Brais B. Neuromuscular disorders with founder effects in French Canada: why, where and how they contribute to the NMD field. *Neuromuscular Disord* 2022; 32: S136.
46. Leckie JN, Joel MM, Martens K, et al. Highly elevated prevalence of spinobulbar muscular atrophy in indigenous communities in Canada due to a founder effect. *Neurol Genet* 2021; 7: e607.
47. Rouleau G, Bélisle-Pipon JC, Birko S, et al. Early career researchers' perspectives and roles in patient-oriented research. *Res Involv Engagem* 2018; 4: 1–15.
48. Leader AE and Aplin AE. From the community to the bench and back again: the value of patient and community engagement in cancer research. *Cancer Discov* 2021; 11: 2135–2138.
49. Richards DP, Cobey KD, Proulx L, et al. Identifying potential barriers and solutions to patient partner compensation (payment) in research. *Res Involv Engagem* 2022; 8: 7.
50. Ianni PA, Samuels EM, Eakin BL, et al. Assessments of research competencies for clinical investigators: a systematic review. *Eval Health Prof* 2021; 44: 268–278.
51. Fagan MB, Morrison CR, Wong C, et al. Implementing a pragmatic framework for authentic patient–researcher partnerships in clinical research. *J Comp Eff Res* 2016; 5: 297–308.