



ENMC collaborative workshops - patient toolkit for applicants and organisers

ENMC, Baarn, The Netherlands

This toolkit has been written for applicants and organisers with the aim to support them in establishing effective patient participation in an ENMC workshop.

1. Introduction

The mission of the 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 optimizing standards of care to improve the quality of life of persons affected with a neuromuscular disorder and their families.

This mission of the ENMC is accomplished through the organisation of workshops (approximately 8-10 per year) which represent our core activity.

The workshops are aimed to be interdisciplinary and encourage a lively debate between the different experts working in the field of clinical and basic NMD research. All participants have an active role at the workshop to achieve the deliverables aimed for. Patients are also invited to secure that the patient's voice is represented as they are living with or are affected by a muscle wasting condition in everyday life. The aim is to establish a long-lasting collaboration between basic scientists, clinicians and patient representatives resulting in improvements in diagnosis, development of treatments and the definition of care pathways for NMDs.

2. Why a patient toolkit?

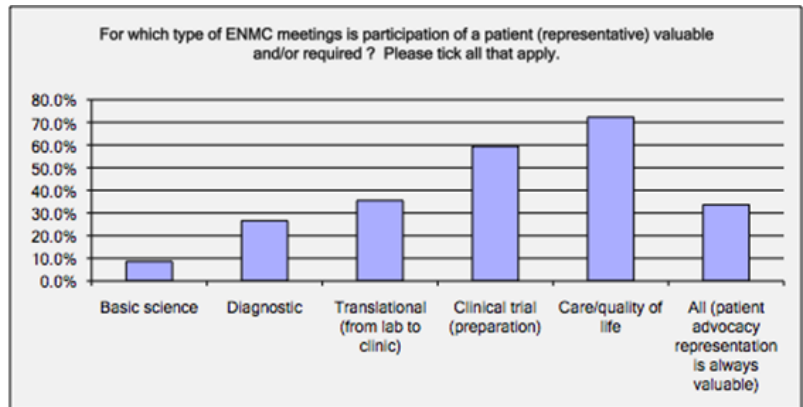
The ENMC was founded in 1991 by a group of patient representatives together with some key scientists and clinicians to accelerate international collaboration and communication with all parties involved. At that time the main aim of the workshops was to define diagnostic criteria and accelerate the pace of research in understanding the underlying genetic and molecular mechanisms of muscle and nerve wasting. This strategic focus slightly changed with the addition of care workshops discussing practical aspects of everyday patient care, e.g. the role of exercise training and management of pain and fatigue. Therefore, patient participation was needed more than ever to share their daily experiences of living with the disease. Since 2014, the participation of one or two patients representatives is mandatory and this should be included in the workshop application.

To stimulate patient participation in the workshops, the ENMC Patient Toolkits were established with help from the ENMC members; i.e. European NMD patient organisations. The following toolkit has been developed as part of this program in order to support workshop applicants and organisers with information and guidance on how to involve patients in the workshop and ensure their voice is being heard and taken into consideration in the discussions. A separate toolkit has been developed for patients, to provide them with information about ENMC workshops and give them guidance on what to expect and how to prepare when planning to take part in a workshop.

3. How do individuals directly or indirectly affected by a neuromuscular condition contribute to scientific and clinical workshops?

An individual directly affected by a neuromuscular condition will be able to bring expertise to the meeting that others without direct involvement will not have. For example, this person will be able to contribute with issues relevant in his/her daily life that clinicians and/or researchers might not have considered.

From a survey conducted in 2012, we have learned that participants of ENMC workshops highly value the participation and input from persons directly or indirectly affected by a neuromuscular disorder. In particular, the input of patients was considered important and highly relevant for those workshops addressing clinical trial readiness aspects and/or quality of life and patient care.



4. Who can be invited to your workshop?

In the first instance people that have a neuromuscular condition themselves should be considered to participate in the workshop, if appropriate. If this is not feasible parents, partners or other close relatives can be invited as patient representatives.

If the participating individual is directly affected by a neuromuscular condition we would also encourage them to be accompanied by a parent, partner or another close relative as they will be aware what it means to live with a neuromuscular condition from day-to-day. If nobody directly affected by a neuromuscular condition is available a representative from a patient organisation can be invited. This person has to be able to represent the patient's voice and should be familiar with the experiences and views individuals living with a neuromuscular condition.

To be able to attend an ENMC workshop the patient representative should be:

- able to talk about the daily life issues and experiences of directly and indirectly affected individuals, e.g. in a short presentation during the workshop
- familiar with current treatment options and standards of care
- able to speak on behalf of the group of patients he or she is representing
- able to speak the English language
- able to broadly follow the scientific discussions
- able to participate in the programme during the 2 ½ day meeting, however is entitled to leave the room to rest or relax at any time
- actively involved in the writing and/or reviewing of the lay report
- physically able to travel to the Netherlands
- either ambulant or be able to use a wheelchair and use the facilities (elevator, restaurant etc.) required.

It is mandatory to invite at least one, but preferably two individuals directly affected by a neuromuscular condition. If possible, patient representatives from two different countries should be invited. This will ensure that patient experiences from different cultural backgrounds will be brought to the attention of the other workshop participants. In case the organiser cannot identify a patient representative the ENMC office can be contacted for assistance. All costs for the patients and their helpers are covered by the ENMC.

5. What is your role as organiser/applicant with regards to patient representation in your workshop?

i. Prior to the workshop:

- Discuss your workshop application with the patient representative that you are considering to participate.
- Ensure the patient is listed on the participants list.
- Ensure the patient representative understands your expectations regarding participation in the workshop (we encourage to use the ENMC toolkit for patients).
- Involve the parent, partner or close relative, if appropriate.
- Include a justification in your application why this person is suited to be invited and what he/she will be contributing.
- The ENMC office will contact the patient representative, to discuss his/her specific requirements with regards to the meeting venue, hotel facilities and dietary.

ii. During the workshop

- Introduce the patient representatives to the other workshop participants and emphasize their role during the weekend. This could be:
 - Giving the individual the opportunity to tell their story (consider a 15 – 30 min presentation),
 - Advocating the needs of the patient groups they are representing
 - Contributing to setting priorities for the direction of future research
 - Contributing in discussions – it is important to explain technical language so the patient representative can follow the discussion
- Writing and/or reviewing the lay report
 - As organiser you are responsible for chairing the discussions - giving everyone a chance to ask questions or make comments. This is particularly important for patient representatives
 - If the patient representative is having difficulties to speak, make sure he/she is being assisted to speak or is represented by the accompanying person
- If the patient is not contributing because he/she seems to be overwhelmed by scientific information, ensure that the individual is comfortable and has been given the opportunity to ask questions and make contributions to the discussion.
- If the patient seems tired or needs a brake, the patient can step out of the meeting for a while and take the time to rest.
- Ask now and then how the patient representative experiences the meeting and whether she/he has any suggestions for improvement, if needed.
- Ensure the other workshop participants understand the research priorities of the patient group.

iii. After the workshop

- Encourage the patient representative to contribute writing and/or reviewing the lay summary (the lay report may be translated and distributed to the lay NMD community via the ENMC patient associations).
- ENMC follows up on the patient representatives to ask them about their experiences during the workshop.
- Discuss what impact their contribution made on the workshop outcome.
- The ENMC office will send a questionnaire to the patient representatives to evaluate their feedback on the workshop programme, opportunities to participate and the meeting facilities.
- Occasionally, the ENMC will interview the patient representative and/or ask for quotes to be published at the ENMC website www.enmc.org or on social media such as Twitter and LinkedIn.
- Patients may be asked by the ENMC office whether they are available for future workshops.

Please let us know if you have any further questions or remarks.

Wishing you a fruitful meeting with a successful patient participation!