ENMC Impact Report 2015

Our year in highlights

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Contents

1 Message from the Chair of the Executive Committee

Marita Pohlschmidt, Chair Executive Committee ENMC

Welcome!

I am pleased to welcome you to this year's *Impact Report* that sets out the great progress and achievements the ENMC has made in 2015.

Our main focus again has been to bring clinicians, researchers and health professionals together to facilitate collaboration and networking of the neuromuscular community.

Last year saw a record number of workshop applications suggesting that the services of the ENMC are in higher demand than ever. And it is good to see the continuous shift from basic science towards workshop topics that are related to prepare the field for clinical trials. Setting up consortia, finding consensus for outcome measures and a collaborative effort to find biomarkers have been amongst the favourite subjects.

From the perspective of an organisation that represents the voice of affected individuals and their families this is very encouraging as the ultimate goal of all our efforts is bringing treatments to the clinic. The number of clinical trials that are currently being carried out has never been greater and in particular for rare conditions this brings its own challenges. I am pleased that the ENMC plays a crucial and unique role in accelerating this *bench-to-bedside* transfer of promising technologies.

Our ambition to increase involvement of people affected by a neuromuscular condition in the workshops has gone from strength to strength and their participation is now an integral part of the workshop planning. More young health professionals are also learning about the ENMC as a greater number have benefitted from our *Young Scientist Program* last year.

Since its establishment 25 years ago the ENMC has been funded through the generous support of European Patient Organisations and I would like to take this as an opportunity to thank them for their continuing commitment over the years. Lately it has also received funding from industry for which I am particularly grateful.

My sincere thanks also go to the members of the Research Committee for their invaluable advice and to the neuromuscular community for putting such an enormous amount of planning and effort into the workshops and making them such a success. And of course my thanks go to the hard working ENMC team in the office in Baarn.

This has been another successful year for the ENMC as the spirit of the organisation continues to strive. But we will keep reaching out because only together we will win the fight and will be able to ensure that everybody affected by a neuromuscular condition has access to effective treatments and eventually cures.

Dr Marita Pohlschmidt,
Chair of the Executive Committee

2 The mission of the ENMC

Almost 25 years ago, a group of scientists and clinicians, together with parents of children affected by a neuromuscular condition, started the ENMC. They had in mind the ultimate goals to improve diagnosis, accelerate the search for effective treatments and improve the quality of life of people with a rare neuromuscular condition. To achieve this goal, it was and still is of utmost importance that experts in this field of orphan disorders share their knowledge and experience and collaborate in research worldwide. The ENMC encourages and facilitates this through the organisation of small-sized, interactive workshops for multidisciplinary groups of researchers and clinicians and persons affected by a neuromuscular condition – a unique concept in the scientific community.

ENMC Mission Statement

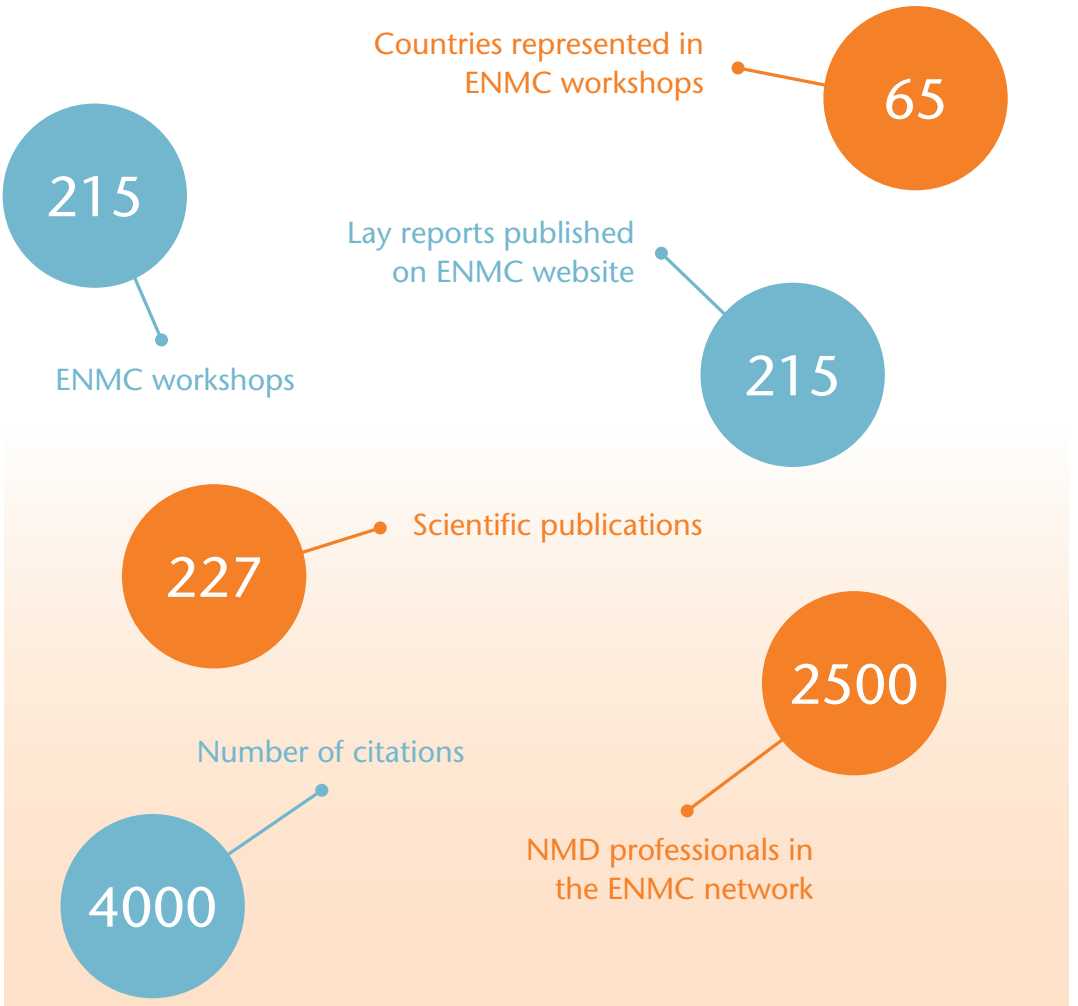
The mission of ENMC is to encourage and facilitate communication and collaboration in the field of neuromuscular research with the aim of improving diagnosis and prognosis, finding effective treatments and optimizing standards of care to improve the quality of life for affected people and their families.

"Connecting people"

3 The impact of ENMC activities over the last two decades

3.1 Number of ENMC workshops, participants and publications

Since its foundation in 1992, 215 ENMC workshops have taken place. This resulted in 215 lay reports published on the ENMC website and 227 scientific publications. We are proud to report that these publications have been cited more than 4000 times. This indicates that the outcomes of ENMC workshops formed the basis for follow-up research to improve diagnosis, treatment and care of persons affected by a neuromuscular condition.



3.2 The ENMC network

The ENMC has now established a network of over 2500 researchers, clinicians and health care professionals working in the field of neuromuscular research and patient care. More and more patients and their representatives join the ENMC network every year. Most members of the network have been involved in one or more ENMC workshops.

The focus of the international collaboration is:

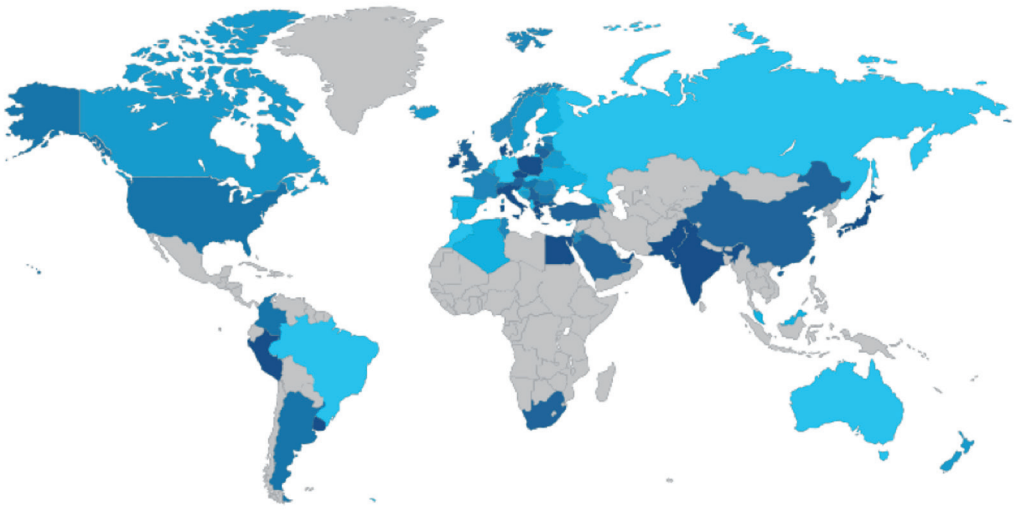
- to avoid fragmentation of research by bringing experts together and facilitating joint efforts;
- to accelerate basic research by sharing biomaterial and animal models;
- to define best practice care guidelines to improve quality of care for every individual with a neuromuscular condition in every country;
- to improve clinical trial readiness and identify the right outcome measures on an international level;
- to initiate and coordinate international clinical trials and to drive the process of bringing new drugs and treatment to the patients;
- to inform the community about the topics and outcomes of ENMC workshops through publication in scientific journals and in lay summaries.

3.3 The gradual globalization of the ENMC

The ENMC was originally founded as a European initiative but, due to its uniqueness, the workshops quickly raised the interest of researchers, clinicians and other health care professionals from all over the world (see map below). With large contributions from ENMC member countries and increasing attendance of experts and patient representatives from the USA and Canada over the last few years, a transatlantic network has now been firmly established.

These 65 countries are given different shades of blue only to distinguish the country borders; the shade of blue does not indicate the number of workshop participants. Countries colored in grey were not represented at an ENMC workshop in the last 20 years.

Over 65 countries are represented in ENMC workshops



4 ENMC workshops in 2015

4.1 A record-breaking number of applications in 2015

With the progress in neuromuscular research and the development of new drugs and therapies for neuromuscular conditions, the need to get together and collaborate is increasing. In 2015, a total of 19 workshop applications were submitted to the ENMC. Compared with the consistent average of 13 applications in the previous five years, this may suggest an increasing demand for ENMC workshops. The fact that we were able to award 50% of the applications in 2015 and previous years indicates the consistent high quality of the applications.

4.2 Summary of ENMC workshops held in 2015

Some of the workshops approved in 2015 were held in 2015, others in 2016. In 2015, a total of six workshops were taking place in both the well-known venue of NH Naarden and the newly appointed venue of Castle Marquette in Heemskerk.
The workshops are listed in the table below.

ENMC workshops in 2015

Date	Workshop No.	Workshop Title
March	210	Towards a European consortium for research and patient clinical management in Spinal and Bulbar Muscular Atrophy (SBMA)
April	211	Development of diagnostic criteria and management strategies for McArdle Disease and related rare glyco(geno)lytic disorders to improve standards of care
May	212	Animal models in congenital muscle diseases (CMD)
September	213	Outcome measures and clinical trial readiness in idiopathic inflammatory myopathies (IIM)
October	214	Establishing an international consortium for gene discovery and clinical research for congenital muscle diseases (CMD)
November	215	Valosin-containing protein (VCP)-related multi-system proteinopathy

4.2.1 Workshop 210 on SBMA

In the first workshop of 2015, the focus was on spinal and bulbar muscular atrophy (SBMA), a very rare, inherited and slowly progressing neuromuscular disease, which only affects men. The disease results in the death of specialized neuronal cells called motor neurons, which control movement of skeletal muscles in the arms and legs. They also control muscles in the face and neck, which are involved in speech, chewing and swallowing. The workshop was a unique opportunity to bring together experts working on SBMA, including Dr Fischbeck from the National Institutes of Health, USA, who first identified the mutation that causes SBMA.

Since muscle weakness is a major problem in SBMA, the hypothesis that exercise may be helpful was tested. Endurance training was found to be efficacious in several muscle diseases but not in SBMA. Other forms of exercise in SBMA are currently being investigated. At the end of the workshop, an official "new European network for SBMA" was established to work on a global SBMA registry, which would facilitate natural history studies, help identify new biomarkers and ensure early recruitment of suitable patients into clinical trials.

For the first time, a patients' forum was organized as an interactive session between patients, clinicians and researchers during the SBMA workshop.

4.2.2 Workshop 211 on McArdle disease

The workshop organisers aimed to select diagnostic criteria for McArdle disease, which is caused by defects in the muscles' glycogen storage system, to define the best practice for management of patients and to plan a strategy for future international clinical trials. Professionals with a wide range of expertises, from neurology, paediatrics, sport and exercise, basic science, respiratory physiology to genetics, and one person affected by the condition joined the workshop. Amongst other results, a recommendation on the beneficial effects of physical activity in McArdle disease was issued, and a quality of life questionnaire specific to this patient population was developed. This workshop was co-funded by EUROMAC, a European network dedicated to McArdle disease and related conditions.

Andrew Wakelin:

"I waited 46 years from first symptoms to receiving guidance on managing McArdle disease. My aim is early diagnosis and immediate provision of best practice information. I have published books and set up a walking course to help people learn good techniques, eliminate bad habits, share experiences and extend their boundaries. The workshop enabled me to make these developments known and to contribute to discussions."

From left to right: *Andrew Wakelin, a patient with McArdle's disease; Dr Ros Quinlivan, clinician and one of the organisers of this workshop; and Dr Renata Scalco, a young physician who received the ENMC young scientist award to attend this workshop.*

4.2.3 Workshop 212 on animal models for congenital muscular diseases (CMD)

CMD constitute a heterogeneous group of rare genetic muscle disorders, the symptoms of which start at birth or within the first few months of life. Over the past decade, the understanding of their defective genetic basis has expanded, with close to 20 genes known to be involved to date. However, this subject still requires the attention of geneticists and clinicians who also came together for a further workshop on genetic discovery and clinical research of CMD (workshop 214, see next page).

In order to further understand the pathogenic mechanisms at play and to assess therapeutic options for these disorders, several animal models were developed in the past, mostly in mice and zebrafish. The objective of workshop 212 was to review the value of the existing animal models for CMD and how their pathology can be extrapolated to human disease. In general, the need to breed animal models on different genetic backgounds to reflect human genetic variability, and to make them available to the scientific community from a central facility was recognized and will be followed up. Furthermore, a registry will be developed which catalogues the different available animal models and provides all known/relevant information to researchers in the field of CMD.

4.2.4 Workshop 213 on idiopathic inflammatory myopathies (IIM)

Participants of the 213th ENMC workshop on idiopathic inflammatory myopathies

IIM comprises a large variety of different rare myositis conditions with an immunological cause of onset. Dependent on where the myositis patients live (country, nearest hospital), their monitoring is done by either rheumatologists, neurologists, dermatologists, internists or paediatricians. As a result, disease evaluation and treatments may vary. The aim of this workshop was to find a consensus between these different medical disciplines regarding the evaluation of response to treatments in the different subgroups of IIM. A strong delegation of patient representatives from different patient organisations (Myositis UK, The Dutch Neuromuscular Diseases Association and AFMTelethon) attended this workshop. They emphasized a focus on neglected symptoms, such as fatigue, and the way these affect social life and work. Mr Ponce, a French myositis patient, pled to develop assessments that are feasible and relevant for patients.

As a result of this workshop, a new study in IIM will be set-up to re-examine the core outcome measures, such as the 6-min walking test, and to develop new measures, such as manual muscle testing, the use of mobile health apps and quality of life questionnaires.

4.2.5 Workshop 214 on genetic discovery and clinical research of congenital muscle diseases (CMD)

This year we had the opportunity to highlight two different but complimentary aspects of one specific group of diseases (CMD) in two different ENMC workshops. Whereas CMD animal models were discussed in workshop 212, expansion of genetic testing for differentiated diagnosis of the various, in some cases very rare, diseases were the topic of workshop 214.

A key barrier to clinical care and therapy development is, in the majority of cases, a lack of genetic diagnosis, due in part to inadequate testing of known genes and in part to currently unknown/unsolved genetic causes. The aim of workshop 214 was to get the experts and stakeholders in this field to work together and establish a new consortium for the study of the genetics of CMD.

Mission statement of CMD consortium:

All individuals with a congenital muscle disorder deserve to have defined the genetic basis of their disease.

The first three aims of the consortium are:

1 the creation of a clinical genetic testing platform for individuals without current access to testing,
2 the implementation of data sharing among existing patient cohorts and
3 the use of a common scale for clinical manifestations.

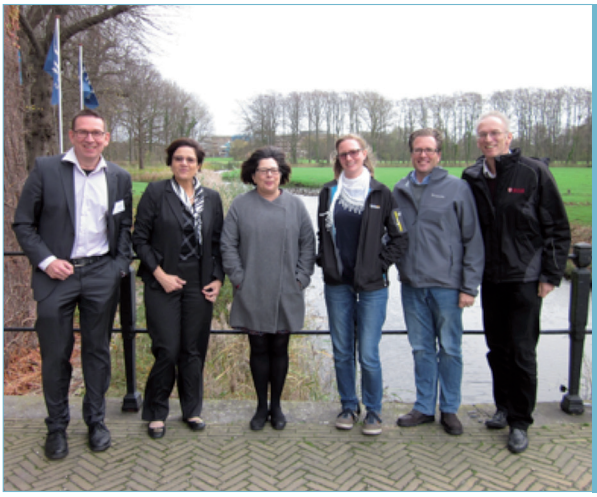
4.2.6 Workshop 215 on VCP-related multi-system proteinopathy

Mutations in valosin-containing protein (VCP) cause an age-related, multi-system degenerative disease which displays variable penetrance and gives rise to four dominant symptoms (phenotypes) within families: myopathy, motor neuron disease, Paget's disease of the bone and frontotemporal dementia (abbreviated IBMPFD). Importantly and distinct to this syndrome, family members may each suffer from a different symptom of the disease.

Much is still unclear about this multi-system disease. Nineteen participants from six different countries with both basic and clinical research expertise in the field of IBMPFD, and two patient representatives came together in Castle Marquette for the 215th ENMC workshop. Prof. Virginia Kimonis, who first recognized IBMPFD as a genetically and clinically distinct syndrome, is excited about the ENMC workshop:

Virginia Kimonis:

"This is the first VCP meeting ever in the world."



Sarah Brumhard (3rd from right), patient representative:

"We have made plans to connect patients with IBMPFD together and the organisers will help me to achieve this."

The workshop helped mutual understanding of the VCP disease's phenotypes and paved the way to standardizing clinical care through a multi-disciplinary clinical team. Ten additional mutations that cause the VCP disease were described, expanding the number to 50. Pre-clinical data from promising therapies applied in mouse models were presented and may form the basis for future clinical trial design.
The patient representative plans to start a Facebook group for the needs and questions of persons who are already diagnosed with VCP disease but are still pre-symptomatic.

Do you also want to organise an ENMC workshop and bring the world top experts with a special focus together for one weekend?

IT TAKES ONLY 6 STEPS TO APPLY:

step 1	step 2	step 3	step 4	step 5	step 6
Submission deadlines	Application guidelines	Application forms	Toolkits for Patient Participation	Young Scientists Program	Review procedure

Check our website: www.enmc.org

5 The ENMC from the perspective of science

Interview with Prof. George Padberg, Research Director of the ENMC

"The scientific impact of ENMC workshops is one of a broad reach."

Professor George Padberg

Picture published thanks to the courtesy of The Dutch Neuromuscular Diseases Association.

Professor George Padberg has been the Research Director of the ENMC for two years. He is an acknowledged neurologist and had a leading role in the discovery of the gene responsible for facioscapulohumeral muscular dystrophy.
George Padberg's vision is to improve the quality of the workshop applications, strengthen collaborations and facilitate participation of patients and young scientists in the workshops, all in close cooperation with the ENMC Office.

Quality stamp

In his position as Research Director, professor Padberg is the chair of the ENMC Research Committee, which is responsible for evaluating the workshop applications on their scientific content, choice of participants, timing and potential impact.

"The Research Committee secures the quality stamp of ENMC workshops."

The ENMC Research Committee and the ENMC Office at the review round in April 2015

Professor Padberg indicates that it is of utmost importance to guarantee excellent quality of the workshops. He is responsible for monitoring the review process of workshop applications and the progress during the ongoing workshops. “The impact of ENMC workshops on the work of my colleagues and myself was and is tremendous, already since the beginning of the ENMC almost 25 years ago. The ENMC made it possible to have specific face-to-face meetings and discuss openly our unpublished data, an environment necessary to start collaborations and thereby speed up research progress.”

Dissemination of knowledge

“The most important scientific impact of ENMC activities is to share knowledge and inform our peers about workshop outcomes. The high number of citations clearly shows that ENMC publications are read frequently within the scientific community. For instance, consensus on guidelines for therapeutic interventions and diagnostic criteria was often achieved at ENMC workshops. Guidelines were labelled at international conferences and through publication as “ENMC guidelines”, confirming again the broad support of the ENMC within the scientific community. This dissemination of guidelines is of utmost importance to ensure uniform and validated procedures in diagnosing and treating patients with a neuromuscular condition across all country borders.” It was also stated by workshop organisers that the ENMC publications are recognised as the most important short term outcome of an ENMC workshop.

> “86% of workshops have been published in a scientific journal.”

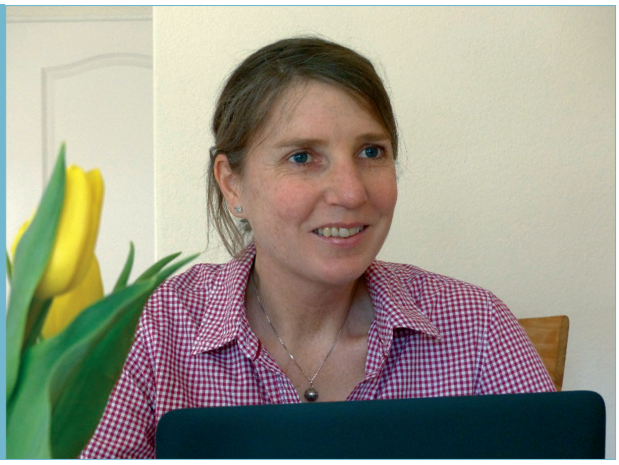
Progress in Research

“We are proud that several disease-specific consortia were established at ENMC workshops. Nowadays, many consortia focus on trial readiness and defining outcome measures in specific neuromuscular diseases. This is a good reflection of the progress being made within clinical research over the years. When the ENMC started about 25 years ago, we were still identifying the genes and mutations responsible for a condition. Now we target these genes with new potential drugs, ready to be developed into clinical trial programs. As we learned from the past, it is vital to reach consensus with all stakeholders on board, including the regulatory authorities and pharmaceutical companies, before we start these huge and costly programs. ENMC workshops are the ideal platform to perform these important steering committee meetings,” George Padberg proudly expressed.

6 The ENMC from the perspective of a patient

Interview with patient representative Mrs Ingrid de Groot

"After the ENMC workshop, I was asked to become a Patient Research Partner (PRP) in the Omeract Project."



Ingrid at home, working behind her laptop.

"Isn't that cool?" Ingrid de Groot responds to me with great enthusiasm. She attended the 213th ENMC workshop on idiopathic inflammatory myositis (IIM), which was held in September, 2015.

Ria Broekgaarden from The Dutch Neuromuscular Diseases Association introduced Ingrid to the ENMC*. As a Chair of the diagnosis myositis workgroup, Ingrid is involved in writing the Myositis Newsletter. She also has translated a MyoNet editorial and is the contact for The Myositis Association (TMA).

Ingrid was one of the 22 workshop participants from 8 different countries with various medical and scientific backgrounds. Together with three other patient representatives from the UK and France, Ingrid formed an excellent delegation of people who have to deal with myositis every day. They presented their experiences and emphasized the needs of patients to the clinicians and researchers in the workshop. Irene Oakley from the Myositis UK group, mother of a child with myositis, for example, made a strong plea that outcome measures should always reflect the needs of patients.

Traditionally, the lay report of an ENMC workshop is written by the scientific organizers of a workshop. Then it is published on the website to inform lay people about the outcomes of the workshop. Ingrid was the first patient in the history of ENMC workshops who took the initiative to write the lay report. She knows exactly what level of information patients can handle and what impact a workshop may have on their future treatment, diagnosis and quality of life.

* The ENMC developed the patient participation programme to reinforce the contribution of patients to the ENMC workshops. Toolkits were developed for both the ***patient representatives*** and the ***researchers/clinicians*** to help them understand what can be expected from each other during the workshops.

"Being the patient's voice during an ENMC workshop... is a great opportunity to *be heard* instead of being talked about."

From left to right: Daniel Ponce from AFMTelethon France, Ingrid de Groot from The Dutch Neuromuscular Diseases Association and Irene Oakley and her husband, founders of the Myositis group UK.

"It's great to be so close to the fire."

For example, she explained in the lay summary what "outcome measures" are and indicated that in addition to the traditional 6 min walk test, additional measures are needed to reflect the quality of life of myositis patients, such as being able to reach the kitchen without support and being able to cook for themselves. "That is why patients, clinicians and researchers welcome the possibilities of technical innovations, for example an app that monitors daily activity and performance at home for months in a row," Ingrid explains.

"Since I am unfortunately no longer able to work due to my condition, I have plenty of time to do some voluntary activities and now I have been asked to become involved in the OMERACT project**, which is dedicated to the development and validation of clinical and imaging outcome measures in rheumatic diseases. At the ENMC workshop, Prof. Ingrid Lundberg from the Karolinska Institute in Sweden asked me to join the project to reflect the needs and wishes of patients in Europe. In May I am going to Canada to attend the OMERACT meeting (bi-annual 4 day conference) and together with other Patient Research Partners, we will ensure that outcome measures in future myositis trials are also relevant for myositis patients. I am assigned to the Special Interest Group Myositis. Our votes are as important as those from the health care professionals and basic researchers who are represented in the OMERACT working groups! I am really grateful that I've been given a chance to take co-ownership over research themes with regard to a rare condition that has such an enormous impact on patients' lives...!"

Interviewed by: Dr Alexandra Breukel,
Managing Director ENMC

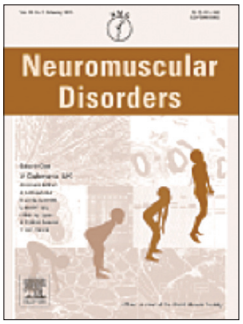
Thanks to the courtesy of the OMERACT office

**Outcome measures in Rheumatology (OMERACT) is an informal international netwerk initiated in 1992 with the aim of improving outcome measurement in rheumatology. OMERACT has built data-driven consensus for many rheumatologic conditions and related disorders, such as myositis. Consensus is reached at the bi-annual conferences involving clinicians, methodologists, regulatory agencies, industry, and patients, who participate as equal partners in the process.

7 Role of ENMC in disseminating information

Publications

A key role for the ENMC is to inform people within the neuromuscular community about the workshops and their outcomes. Clinicians and researchers obtain this knowledge via scientific publications, which have been cited more than 4000 times.



International conferences

In addition, the ENMC started to focus on raising its profile towards the next generation of young researchers and clinicians who are developing their expertise in the field of neuromuscular disorders. We anticipate this to be the new generation of workshop applicants and organisers, who will continue the work of previous workshops and may establish new consortia.

The ENMC team attended the World Muscle Society Congress held in October 2015 in Brighton (UK) to meet scientists and clinicians at this international conference.

Social media

Over the years, the ENMC office developed several ways to disseminate information to lay people. Through publication of the lay reports on our website and messages on social media like Twitter and LinkedIn, we try to reach affected people and their families worldwide.

www.enmc.org

Innovations

Two of the most important innovations in 2015 were the development of e-newsletters and the make-over of the ENMC website. These innovations were successful: the e-newsletters were read by one third of our network and the ENMC website was frequently visited by people from and outside the ENMC network. Google analytics shows that 13.500 sessions from 11.250 visitors took place from 1 January until 31 December 2015. Of these visitors, 83% are new to the website. Most of them originate from the USA (26%), followed by the Netherlands (11%), UK (6%), France (5%), Italy (4%), Germany (4%) and China (4%), and others are from countries like Poland, Russia, Brazil, Australia and India. Thus, we have a worldwide coverage.

8 Resources and Financial Management in 2015

8.1 The ENMC Company Forum

In the last decade, the number of clinical trials for neuromuscular disorders increased steadily. For some diseases, the first treatments are expected to become available to patients in the next few years. However, there is still much to do:

- The existing technologies for diagnosis require optimisation.
- Clinical trial readiness needs further improvement.
- International consensus of best practice care guidelines needs to be reached and implemented for all rare neuromuscular conditions.

In 2015, the ENMC started an initiative called "ENMC Company Forum", which is a platform for pharmaceutical and biotech companies that have neuromuscular disorders as one of their areas of interest. Members of the ENMC Company Forum support us to respond to the increasing demand for ENMC workshops and thereby facilitate collaboration of experts working in the field of neuromuscular disease worldwide.

The ENMC Company Forum was launched on 1 October 2015 in Brighton, with representation of the members of the ENMC Company Forum and of the ENMC Executive and Research Committees. Members of the ENMC Company Forum co-sponsor the general activities of the ENMC and the young scientist programme. They will be associated with the mission and the heritage of the ENMC.
The relationship between the ENMC and companies is regulated by the established international guidelines regarding inducements and sponsorship.

> If you have any questions regarding the ENMC Company Forum and/or your organisation is interested in becoming a member, please contact us at enmc@enmc.org or +31-35-5480482.

8.2 Financial summary 2015

Annual accounts for the year 2015 were compiled in accordance with accounting principles for non-profit organisations generally accepted in the Netherlands (RJ640). The financial accounts are drawn up in Euros.

In the summary table on the next page, the overall income and expenses over the year 2015 are summarized in comparison with the figures for the financial year 2014. The difference in figures between 2014 and 2015 is the result of a change in the budgeting system, which was decided at the ENMC Executive Committee Meeting in May 2015. Details are given in the annual report 2015, which can be downloaded from the website.

Statement of income and expenses for the year 2015 in Euros (€)		
	2015	2014
INCOME		
Member contributions	210.000	210.000
Associated member contributions	5.000	5.000
Company Forum contributions	48.333	-
Other contributions	40.309	88.690
Total income	303.642	303.690
EXPENSES		
Personnel expenses	115.529	104.920
Rental expenses	11.346	11.000
Activity (workshop) expenses	196.272	80.401
Organisational expenses	50.028	40.732
Total Expenses	373.925	237.053
Operating result	-70.283	66.637
Interest income	3.085	3.749
Net result	-67.198	70.386
APPROPRIATION OF RESULTS		
Continuity reserve	200.000	-
Other free reserves	-267.198	70.386
	-67.198	70.386
CASH AT BANKS ON 31 DECEMBER	518.654	427.031

Opinion of the auditors

The ENMC accountants have verified and approved the annual accounts. For a full PDF version of the annual accounts report of 2015, please visit the ENMC website.

www.enmc.org

9 Governance 2015

The ENMC was founded on 24 November 1992 under Dutch law. The foundation is supported by financial contributions of nine European patient organisations for neuromuscular disorders and other related organizations. The statutory location is in Baarn in the building of The Dutch Neuromuscular Diseases Association.

9.1 The ENMC Executive Committee

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

Composition of the ENMC Executive Committee on 31 December 2015:
Dr A. Ambrosini (Italy)
Dr A. Méjat (France)
Dr A. von Moers (Germany)
Dr M. Mootz (The Netherlands)
Dr M. Pohlschmidt (Chair, United Kingdom)
Dr J. Rahbek (Denmark)
Dr E. Sterrenburg (The Netherlands)
Dr R. Willmann (Vice-Chair, 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 2015:
Prof. Dr G.P. Comi (Italy)
Dr M. Eagle (United Kingdom)
Dr D. Hilton-Jones (United Kingdom)
Dr P. Laforêt (France)
Prof. Dr A. Oldfors (Sweden)
Prof. Dr G. Padberg (Chair, the Netherlands)
Prof. Dr M.A. Rüegg (Switzerland)
Prof. Dr U. Schara (Germany)
Prof. Dr B. Schoser (Germany)
Prof. Dr P. Shaw (United Kingdom)
Dr B. Talim (Turkey)

9.3 The ENMC Office

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

ENMC Office staff on 31 December 2015:
Dr A. Breukel (Managing Director)
Mrs A. Zittersteijn (Operational Manager)
Prof. Dr G. Padberg (Research Director)
Ms J. Tiel-Groenestege (Workshop Assistant)

10 A special thanks to all our members and supporters

Thanks to the continuous support of the nine European patient organizations, the ENMC is able to facilitate and organise an average of eight workshops per year. With support from additional partner organizations, such as condition-specific associations and members of the ENMC Company Forum, we are also able to invite participants from non-ENMC countries and facilitate the attendance of young scientists and patient representatives.

ENMC full and associated members:

Finnish Neuromuscular Disorders Association

Telethon

SPIERZIEKTEN NEDERLAND

Deutsche Gesellschaft für Muskelkranke e.V. DGM

Muscular Dystrophy UK
Fighting muscle-wasting conditions

MUSKELSVINDFONDEN

PRINSES BEATRIX SPIER FONDS
KRACHTIG TEGEN SPIERZIEKTEN

AFMTÉLÉTHON
INNOVER POUR GUERIR

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

Members of the Company Forum:

genzyme
A SANOFI COMPANY

Amicus Therapeutics

PTC THERAPEUTICS

santhera Pharmaceuticals

Workshop-specific sponsors:

Kennedy's Disease Association

A Foundation BUILDING STRENGTH For Nemaline Myopathy

MYOSITIS UK

cureCMD congenital muscular dystrophy

EUROMAC

Funded by the Health Programme of the European Union

Roche

myotubular trust FINDING STRENGTH

LFB L'ENGAGEMENT ÉTHIQUE

aTyr Pharma

The Myositis Association

MedImmune

AstraZeneca

11 Looking forward to 2016

Nine workshops are scheduled for 2016 (see table below). Two review rounds are planned for 2016 (spring and autumn).

The workshops that are selected will be planned for the second half of 2016 and the first half of 2017.

Workshop programme 2016 (www.enmc.org)

Workshop no. / date	Topic	Workshop leaders
Workshop no. 216 January 15 - 17	Clinical trial readiness for FKRP related muscular dystrophies	I. Richard (France), S. Cirak (Germany), J. Vissing (Denmark), J. P. Laurent (USA)
Workshop no. 217 January 29 - 31	RYR1-related myopathies	J. Dowling (USA), H. Jungbluth (UK), A. Ferreiro (France), F. Muntoni (UK)
Workshop no. 218 February 19 - 21	Eight years on, revisiting the Consensus Statement for Standards of Care in Spinal Muscular Atrophy (SMA)	T. Sejersen (Sweden), R. Finkel (USA), E. Mercuri (Italy)
Workshop no. 219 April 29 - May 1	Titinopathies - international database of TTN mutations and phenotypes.	P. Hackman (Finland), B. Udd (Finland), C. Bönnemann (USA), A. Ferreiro (France)
Workshop no. 220 May 27 - 29	The 2nd ENMC Workshop on dystroglycan and the dystroglycanopathies	S. Winder (UK), S. Brown (UK)
Workshop no. 221 June 10 - 12	Foot surgery in Charcot-Marie-Tooth disease (CMT)	M. Reilly (UK), D. Pareyson (Italy), D. Singh (UK), J. Burns (Australia)
Workshop no. 222 July 1 - 3	Myotonic dystrophy, developing a European consortium for care and therapy	H. Lochmüller (UK), B. van Engelen (The Netherlands), B. Schoser (Germany), G. Bassez (France)
Workshop no. 223 September 16 - 18	AAV Microdystrophin gene therapy: trial ready for Duchenne muscular dystrophy (DMD)	G. Dickson (UK), F. Mavilio (France), C. Le Guinier Blanvillain (France), D. Ribeiro, (UK)
Workshop no. 224 October 14 - 16	Clinicopathological classification of immune-mediated necrotizing myopathies	Y. Allenbach (France), O. Benveniste (France), A. Mammen (USA), W. Stenzel (Germany)

Goals for 2016

For 2016, our goal is to focus on our core activity, i.e. facilitating and organizing the ENMC workshops, and on consolidating the initiatives that we started in 2015.

Patient involvement: We want to ensure patient partnership in the ENMC workshops and broaden the dissemination of information to lay persons.

Finances: Our aim is to increase our budget through workshop-specific donations and the ENMC Company Forum in order to meet the increased demand for ENMC workshops, continue the globalization of ENMC workshops and facilitate the participation of young scientists and patient representatives in the ENMC network.

Notable upcoming events: With regard to international conferences, ENMC ambassadors will be present at the upcoming Myology conference in Lyon, France, The Dutch Neuromuscular Diseases Association congress in Eindhoven, The Netherlands and the World Muscle Society meeting in Granada, Spain.

Preview from ENMC workshop May 2016, Neil Bevan and his wife attend the workshop on dystroglycanopathies as patient representatives.

Colophon

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