ENMC Impact Report 2015

Our year in highlights
Contents

1 Message from the chair of the Executive Committee 3
2 The mission of the ENMC 4
3 The impact of ENMC activities over the last two decades 5
   3.1 Number of ENMC workshops, participants and publications 5
   3.2 The ENMC network 6
   3.3 The gradual globalization of the ENMC 6
4 ENMC workshops in 2015 7
   4.1 A record-breaking number of workshop applications in 2015 7
   4.2 Summary of ENMC workshops held in 2015 7
5 The ENMC from the perspective of science 12
   Interview with Prof. George Padberg, Research Director of the ENMC
6 The ENMC from the perspective of a patient 14
   Interview with patient representative Mrs Ingrid de Groot
7 Role of ENMC in disseminating information 16
8 Resources and financial management 2015 17
   8.1 The ENMC Company Forum 17
   8.2 Financial summary 2015 17
9 Governance 2015 19
   9.1 The ENMC Executive Committee 19
   9.2 The ENMC Research Committee 19
   9.3 The ENMC Office 19
10 A special thanks to all our members and supporters 20
11 Looking forward to 2016 22
1 Message from the Chair of the Executive Committee

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
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.

Over 65 countries are represented in ENMC workshops

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.
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

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

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.
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)

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.”
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:

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

Check our website: www.enmc.org
“The scientific impact of ENMC workshops is one of a broad reach.”

*Professor George Padberg*

---

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.”
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.

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.

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

“Being the patient’s voice during an ENMC workshop... is a great opportunity to be heard instead of being talked about.”

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.

“It’s great to be so close to the fire.”

“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

Thanks to the courtesy of the OMERACT office

**Outcome measures in Rheumatology (OMERACT) is an informal international network 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.
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.

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.

Follow, like and retweet us!

www.enmc.org
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 (Rij640). 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.
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.

## Statement of income and expenses for the year 2015 in Euros (€)

<table>
<thead>
<tr>
<th></th>
<th>2015</th>
<th>2014</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>INCOME</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Member contributions</td>
<td>210.000</td>
<td>210.000</td>
</tr>
<tr>
<td>Associated member contributions</td>
<td>5.000</td>
<td>5.000</td>
</tr>
<tr>
<td>Company Forum contributions</td>
<td>48.333</td>
<td>-</td>
</tr>
<tr>
<td>Other contributions</td>
<td>40.309</td>
<td>88.690</td>
</tr>
<tr>
<td><strong>Total income</strong></td>
<td>303.642</td>
<td>303.690</td>
</tr>
<tr>
<td><strong>EXPENSES</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Personnel expenses</td>
<td>115.529</td>
<td>104.920</td>
</tr>
<tr>
<td>Rental expenses</td>
<td>11.346</td>
<td>11.000</td>
</tr>
<tr>
<td>Activity (workshop) expenses</td>
<td>196.272</td>
<td>80.401</td>
</tr>
<tr>
<td>Organisational expenses</td>
<td>50.028</td>
<td>40.732</td>
</tr>
<tr>
<td><strong>Total Expenses</strong></td>
<td>373.925</td>
<td>237.053</td>
</tr>
<tr>
<td>Operating result</td>
<td>-70.283</td>
<td>66.637</td>
</tr>
<tr>
<td>Interest income</td>
<td>3.085</td>
<td>3.749</td>
</tr>
<tr>
<td><strong>Net result</strong></td>
<td>-67.198</td>
<td>70.386</td>
</tr>
<tr>
<td><strong>APPROPRIATION OF RESULTS</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Continuity reserve</td>
<td>200.000</td>
<td>-</td>
</tr>
<tr>
<td>Other free reserves</td>
<td>-267.198</td>
<td>70.386</td>
</tr>
<tr>
<td></td>
<td>-67.198</td>
<td>70.386</td>
</tr>
<tr>
<td><strong>CASH AT BANKS ON 31 DECEMBER</strong></td>
<td>518.654</td>
<td>427.031</td>
</tr>
</tbody>
</table>

[www.enmc.org](http://www.enmc.org)
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 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
- Muscular Dystrophy UK (fighting muscle-wasting conditions)
- MUSKELSVINDFONDEN
- PRINSES BEATRIX SPIERFONDS (krachtig tegen spierziekten)
- AFMTELETHON (innover pour guérir)
- fsrmm (fondation suisse de recherche sur les maladies musculaires schweiz. stiftung für die erforschung der muskelkrankheiten)

Members of the Company Forum:
Workshop-specific sponsors:
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)

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

Text editors
Alexandra Breukel
Raffaella Willmann
Ellen Sterrenburg
Annelies Zittersteijn

English translator
Sonja von Aulock

Lay out
Vormgevers Arnhem, The Netherlands

Printing
Year of printing 2016. Printing by DrukwerkDeal