



Our year in highlights

ENMC Impact Report 2014

EUROPEAN
NEURO
MUSCULAR
CENTRE

Contents

Message from the Chair Executive Committee	3
The ENMC organisation	4
The impact of ENMC over the last two decades	5
ENMC workshops in 2014	8
Patient Participation in 2014	11
Testimonials	12
Young Scientist Programme in 2014	13
Increased ENMC visibility in 2014	14
Resources and Financial Management in 2014	16
Acknowledgements	18
Looking Forward to 2015	19

Message from the Chair of the Executive Committee



Marita Pohlschmidt, Chair
Executive Committee ENMC

Welcome!

We are delighted to report on the achievements and activities the ENMC has made during the last year in encouraging researchers, clinicians and patients from all over the world to network and collaborate.

In 2014 we organized six workshops on highly relevant topics such as biomarkers, standards of care and clinical trial readiness. In total more than 150 neuromuscular experts from more than 20 countries came together for a weekend in the Netherlands to find pressing solutions for the issues they had on their agenda.

At the beginning of the year we welcomed our new Managing Director Dr Alexandra Breukel into the team in Baarn. Alexandra has been very keen on giving the ENMC more visibility and ensuring that experts working in the neuromuscular field, especially the young generation, are aware of the opportunities our organisation offers. The team attended conferences and other meetings to successfully increase the reach and engagement within the neuromuscular community.

And they had scientific support by our new Chair of the Research Committee, Professor George Padberg who also took up this position at the start of 2014. We are very pleased to have him on board and are grateful for his enthusiasm for everything the ENMC stands for.

Last year a restart was made with an important programme aiming to increase participation

of people with a neuromuscular condition in the workshops. Ensuring that the views of the patients are heard and considered by researchers and clinicians is crucial for the development of treatments and the definition of best practice care guidelines. This is very close to my heart and I was pleased that our efforts received such great welcome.

The ENMC does not receive any statutory funding and I would like to take this as an opportunity to thank the patient organisations and other supporters especially from industry for their continued support over the years.

My sincere thanks also go to the dedicated researchers, clinicians and patient representatives for their time and effort and for their willingness to give up their weekends to take part in the workshops. And of course my thanks go to the hard working ENMC team in Baarn.

The concept of the ENMC can only reach its full potential if we work together to ensure that one day there are effective treatments and eventually cures for everybody with a neuromuscular condition.

A handwritten signature in dark ink, reading 'Marita Pohlschmidt'.

Dr. Marita Pohlschmidt

The ENMC organisation

The ENMC facilitates collaboration and communication in the field of neuromuscular research through the organisation of interdisciplinary workshops for expert scientists, clinicians and health professionals. The workshops provide an opportunity for the scientific community to share their knowledge and experience to improve diagnosis and prognosis, to drive progress in basic and translational research and to establish new consortia. The concept of the ENMC is **unique** in the world with the ultimate goal in mind. This is to accelerate the search for effective treatments and improving quality of life for people with a neuromuscular condition and their families.

The ENMC was founded November 24, 1992 under Dutch law. The foundation is supported through financial contribution of a number of European patient organisations for neuromuscular disorders and other related organizations. The statutory location is in Baarn in the building of the Association of Muscle Disorders in the Netherlands.

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

ENMC Office staff per December 31, 2014:

Dr. A. Breukel
Mrs. A. Zittersteijn
Prof. Dr. G. Padberg
Mrs. D. Dute

Composition of the Executive Committee per December 31, 2014:

Dr. M. Pohlschmidt (Chair, United Kingdom)
Dr. R. Willmann (vice-Chair, Switzerland)
Dr. A. Ambrosini (Italy)
Mr. J.F. Malaterre (France)
Dr. A. von Moers (Germany)

Dr. M. Mootz (The Netherlands)
Dr. J. Rahbek (Denmark)
Dr. E. Sterrenburg (The Netherlands)
Prof. Dr. G. Padberg (The Netherlands, non-voting).

Composition of the Research Committee per December 31, 2014:

Prof. Dr. G. Padberg, Chair and Research Director (the Netherlands)
Prof. G.P. Comi (Italy)
Prof. M.A. Rüegg (Switzerland)
Prof. A. Ferlini (Italy)
Dr. D. Hilton-Jones (United Kingdom)
Dr. P. Laforet (France)
Prof. Dr. A. Oldfors (Sweden)
Prof. Dr. B. Schoser (Germany)
Dr. B. Talim (Turkey)
Prof. Dr. B. Udd (Finland)
Prof. Dr. U. Schara (Germany)

The ENMC Network

The ENMC has established a network of over 2500 researchers, clinicians and health professionals working in the field of neuromuscular research and patient care. Each member has been involved in one or more ENMC workshops or alternatively has an interest to receive updates on the organisation's activities.

The focus of the international collaboration is:

- to accelerate basic and clinical research
- to define best practice care guidelines to ensure every individual in the world with a neuromuscular condition receives the highest standards of care
- to improve clinical trial readiness on an international level
- to initiate and coordinate international clinical trials and to drive the process for bringing new drugs and treatment to the market
- to inform the community about the topics and outcome of ENMC workshops through the publication in peer reviewed scientific journals and in lay summaries

The impact of the ENMC over the last two decades

To understand the impact the ENMC workshops have made over time we looked at three different areas that we analyzed over a period of 20 years (with data from 1995 up to 2014):

1. The gradual globalization of the ENMC
2. Descriptive statistics regarding workshops, members in the ENMC network, publications, lay reports, citations in peer reviewed journals
3. The scientific impact of the ENMC

This is, of course, only a starting point but will be used as a baseline reference to monitor our achievements in the future.

1. The gradual globalization of the ENMC

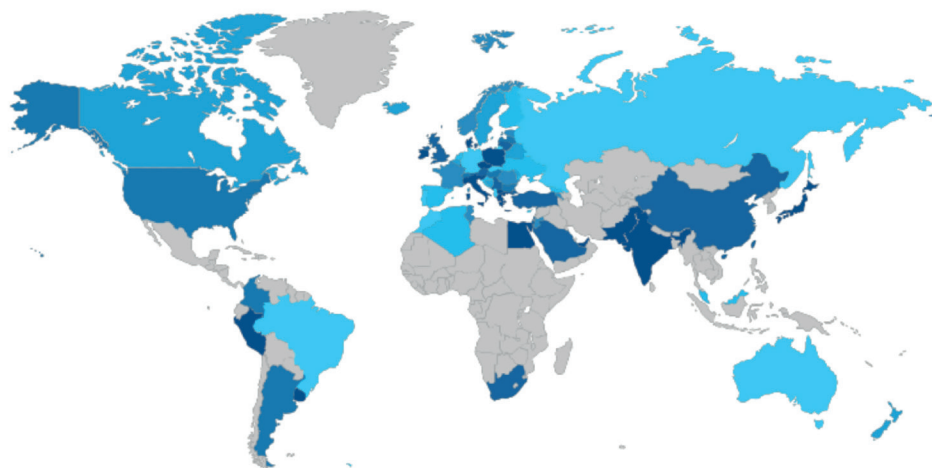
The ENMC was originally founded as a European initiative but due to its uniqueness, the work-

shops quickly raised the interest of researchers, clinicians and other health professionals from all over the world.

The ENMC is now a truly international organization including experts from all over the world. This has helped to put our mission into practice and we can now expect to achieve our objective to finding effective treatments and eventually cures with greater speed than 20 years ago. In total 65 countries had at least one representative in an ENMC workshop over the last 20 years as you can see below in the worldwide map.

With this large contribution from ENMC member countries and increasing attendance of researchers, clinicians and patient representatives from the USA and Canada over the last few years, a cross-Atlantic network has been firmly established.

Over 65 countries are represented in ENMC workshops

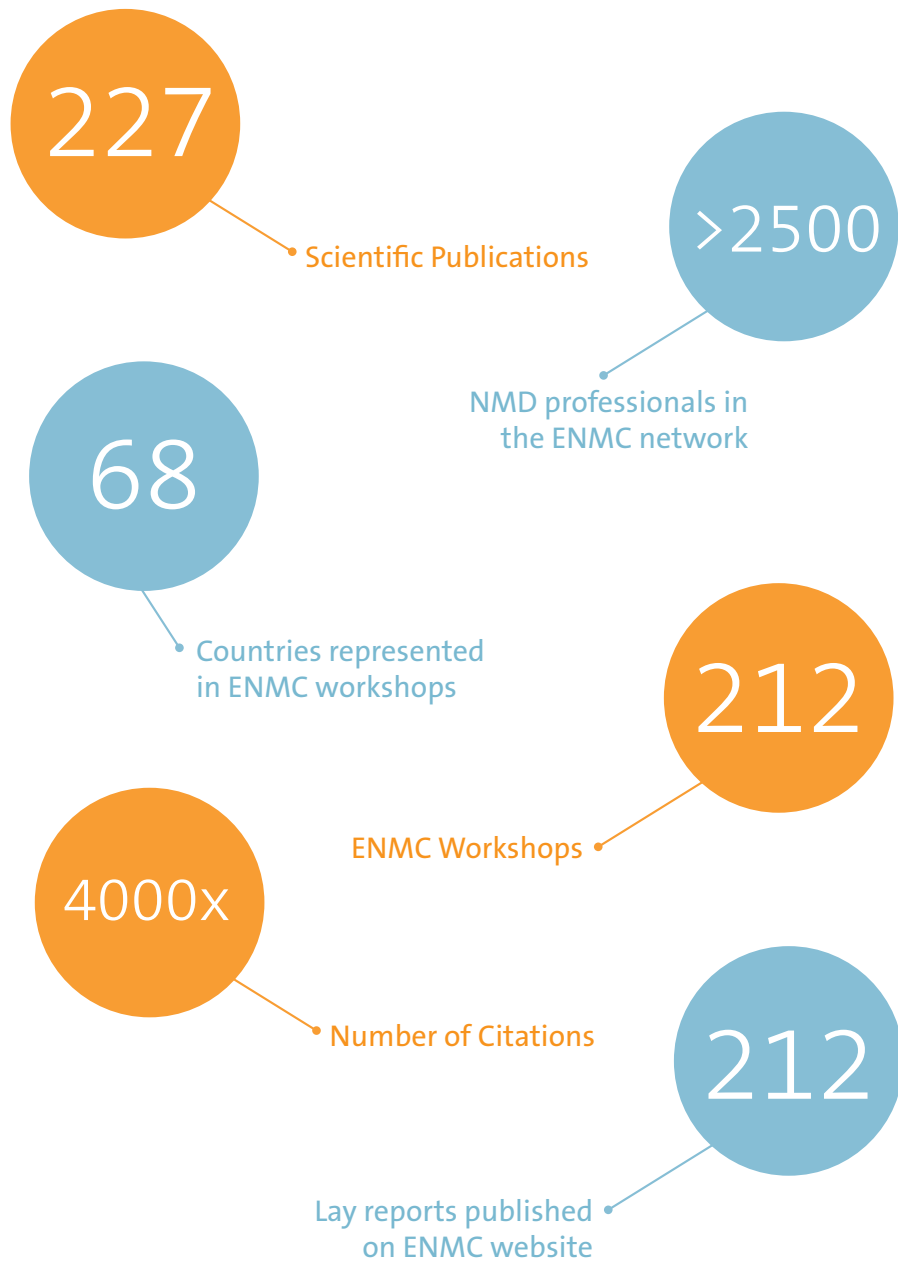


These 65 countries are colored in different blue shades only to distinguish the country borders, the shade of blue does not indicate the number of workshop participants meaning.

2. Descriptive statistics

Since its foundation in 1992, 212 ENMC workshops with more than 2500 participants from 68 countries worldwide have taken place. This resulted in 212 lay reports published on the ENMC website and 227

scientific publications. These include the 118 full reports published in the Journal of Neuromuscular Disorders and other publications. Over the last 20 years, workshop publications have been cited over more than 4000 times in scientific peer reviewed journals.



Status June 2015

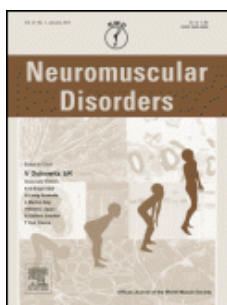
3. Scientific impact

Our workshop archive goes back to 1995, therefore we have analysed a 20-year period (from 1995 to 2014) to measure the scientific impact of ENMC workshops. There is a requirement for the workshop organizer to publish a report of an ENMC workshop in the 'Journal of Neuromuscular Disorders'.

Of 168 workshops held in the period 1995-2014, 118 reports were published resulting in over more than 70% coverage. In the 5-year periods 2010-2014, 2005-2009 and 2000-2004, similar results were obtained; 74%, 72 % and 72% respectively. The high and consistent numbers reflect the scientific value of the ENMC workshops for researchers and clinicians in the NMD field.

In the literature analysis we searched for all publications with "ENMC" or "European Neuromuscular Centre" in the title, funding, acknowledgements or in the keywords. A total of 227 papers fulfilled one of these criteria, double-counts have been eliminated. This list of 227 papers included the already mentioned 118 full reports in the Journal of Neuromuscular Disorders and the remaining papers have been published in many other journals, such as Journal of Medical Genetics, Journal of Neurology, Neurosurgery and Psychiatry, Neuropediatrics, Human Molecular Genetics, American Journal of Human Genetics, Annals of Neurology etc.

"70% of workshops reports are published"



Workshop Reports

2015

208th ENMC International Workshop: Formation of a European Network to develop a European data sharing model and treatment guidelines for Pompe disease Naarden, The Netherlands, 26-28 September 2014

Benedikt Schoser, Pascal Laforêt, Michelle E. Kruijshaar, Antonio Toscano, Pieter A. van Doorn, Ans T. van der Pijeg, European Pompe Consortium (EPOC)
Vol. 25, Issue 6, p674-678

[Abstract](#) | [Full-Text HTML](#) | [PDF](#)

209th ENMC International Workshop: Outcome Measures and Clinical Trial Readiness in Spinal Muscular Atrophy 7-9 November 2014, Heemskerk, The Netherlands

Richard Finkel, Enrico Bertini, Francesco Muntoni, Eugenio Mercuri, ENMC SMA Workshop Study Group
Vol. 25, Issue 7, p593-602

[Abstract](#) | [Full-Text HTML](#) | [PDF](#)

207th ENMC Workshop on chronic respiratory insufficiency in myotonic dystrophies: Management and implications for research, 27-29 June 2014, Naarden, The Netherlands

VA. Sansone, C. Gagnon, participants of the 207th ENMC Workshop
Vol. 25, Issue 5, p432-442

[Abstract](#) | [Full-Text HTML](#) | [PDF](#)

203rd ENMC International workshop: Respiratory pathophysiology in congenital muscle disorders: Implications for pro-active care and clinical research 13-15 December, 2013, Naarden, The Netherlands

A. Rutkowski, M. Chatwin, A. Koubourilis, B. Fauroux, A. Simonds, for the CMD Respiratory Physiology Consortium
Vol. 25, Issue 4, p353-358

[Abstract](#) | [Full-Text HTML](#) | [PDF](#)

205th ENMC International Workshop: Pathology diagnosis of idiopathic inflammatory myopathies Part II 28-30 March 2014, Naarden, The Netherlands

Jan L. De Bleecker, Boel De Paepe, Eleonora Aronica, Marianne de Visser, ENMC Myositis Muscle Biopsy Study Group, Anthony Amato, Eleonora Aronica, Olivier Benveniste, Jan De Bleecker, Onno de Boer, Boel De Paepe, Marianne de Visser, Maren Dimachkie, Romain Gherardi, Hans Himar Goebel, David Hilton-Jones, Janice Holton, Ingrid E. Lundberg, Andrew Mammen, Frank Mastaglia, Ichizo Nishino, Elisabeth Rushing, Henrik Daa Schroder, Duygu Selcen, Werner Stenzel
Vol. 25, Issue 3, p256-272

[Abstract](#) | [Full-Text HTML](#) | [PDF](#) | [Supplemental Materials](#)

204th ENMC International Workshop on Biomarkers in Duchenne Muscular Dystrophy 24-26 January 2014, Naarden, The Netherlands

Alessandra Ferlini, Kevin M. Flanigan, Hanns Lochmuller, Francesco Muntoni, Peter A.C. 't Hoen, Elizabeth McNally
Vol. 25, Issue 2, p194-198

[Abstract](#) | [Full-Text HTML](#) | [PDF](#)

Measuring clinical effectiveness of medicinal products for the treatment of Duchenne muscular dystrophy

Stephen Lynn, Annemieke Aartsma-Rus, Kate Bushby, Pat Furlong, Nathalie Goemans, Annamaria De Luca, Anna Mayhew, Craig McDonald, Eugenio Mercuri, Francesco Muntoni, Marita Pohlschmidt, Jan Verschuuren, Thomas Voit, Elizabeth Vroom, Dominic J. Wells, Volker Straub
Vol. 25, Issue 1, p86-105

[Abstract](#) | [Full-Text HTML](#) | [PDF](#)

ENMC workshops in 2014

Facts & Figures

In 2014, six workshops were organised and have successfully achieved their aims and deliverables. This year a variety of topics were discussed, including the identification of biomarkers for Duchenne muscular dystrophy, the pathology and diagnosis of idiopathic inflammatory myopathies, defining best standards of care for adult men with Duchenne, management and implications of chronic respiratory insufficiencies in myotonic dystrophies, the formation of a Pompe consortium and outcomes and clinical trial readiness for spinal muscular atrophy. The workshops are listed in the table below and the lay summaries can be downloaded from the website.

ENMC workshops in 2014		
Date Workshop	Workshop nr	Title Workshop
February	204	Biomarkers in DMD
April	205	Pathology Diagnosis of idiopathic inflammatory myopathies
May	206	Adults with DMD : Care for a novel group of patients
June	207	Chronic respiratory insufficiency in myotonic dystrophies: management and implications for research
September	208	Formation of a European network to develop a European data- sharing model and treatment guidelines for Pompe disease
November	209	SMA outcomes and clinical trial readiness



ENMC Workshop 204 on Biomarkers in Duchenne Muscular Dystrophy in January 2014

Impact

The six ENMC workshops that took place in 2014 achieved important goals:

- Establishment of international collaborations or consortia
- Reaching consensus on new care guidelines
- Defining new outcome measures for clinical trials

Please find below quotes from workshop organisers that illustrate the importance of the workshops to facilitate international collaboration and give the scientific community a platform to discuss relevant and timely topics and together find solutions. Lay reports of all six workshops have been published at the ENMC website (www.enmc.org) and for five workshops the scientific report has already been published in the Journal of Neuromuscular Disorders. We expect the last report to be published soon.

Quote from Alessandra Ferlini on the 204th ENMC workshop on Biomarkers for Duchenne Muscular Dystrophy:

“The outcomes of the workshop articulate a variety of collaborative actions. One of these is establishing an EU–USA collaboration by sharing data/samples/methods to maximize success in the biomarker identification and validation, harmonizing phenotype definition”.

Prof Dr. Ans van der Ploeg said about the 208th ENMC workshop on Pompe Disease:

“A European Pompe network was formed. The network is initially comprised of the participants of the meeting, but will over time be expanded to involve more European countries, and later possibly also experts from countries outside Europe”.



ENMC workshop 208 on Pompe's disease in September 2014

**Prof. Dr. Jan de Bleecker about the 205th ENMC workshop
on pathology diagnosis of idiopathic inflammatory myopathies:**

"We reached consensus on new international guidelines for the diagnosis of idiopathic inflammatory myositis patients and published them in NMD. This is a next step in agreement regarding biopsy reading between various sub-specialties (pathology, neurology and rheumatology)."



ENMC workshop 205 on pathology diagnosis of IIM in April 2014

Besides the workshops that represent the core activity of the ENMC, the focus this year was also on implementing two further programs that are close to the heart of the ENMC.

Patient participation in the workshops has always been important to the ENMC since it was founded in 1992. In 2014, this area received particular attention and first successes can already be reported with more lay people than ever participating last year. In addition, a second program, the **Young Scientist Award Programme** started in collaboration with Genzyme in 2013 and saw its first young scientist attending an ENMC workshop in 2014.

Patient participation in ENMC workshops

Facts & Figures

The most important accomplishment of the ENMC in 2014 was the increased patient participation in ENMC workshops. The number of patients and/or representatives that participated in the 2014 workshops was increased by more than 60%.

↑
60%

Patient Participation in 2014

At the time the ENMC was founded, 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. Five years ago, this strategic focus changed slightly with the addition of practical care workshops discussing aspects of everyday patient care, e.g. the role of exercise training and management of pain and fatigue. It became increasingly crucial to give patients a greater voice in the workshops so that they could share their daily practical experiences of living with the condition with the scientific community. Prior to this it was mainly representatives from patient associations who participated in workshops to represent the patients' voice.

A patient's toolkit has been developed as part of this programme to support workshop applicants and organisers with information and guidance on how to involve patients in the workshop and ensure that their voice is being heard and considered in the discussions. A separate toolkit has been developed to provide patients with information about ENMC workshops and give them guidance on what to expect and how to prepare for taking part in a workshop. Both toolkits can be found on the ENMC website.

We also amended our general workshop guidelines which now state that participation of at least two patients and/or patient representatives is mandatory in every workshop.



A great example of how patient participation was successfully implemented is the 206th ENMC workshop on care for a novel group of patients, adult men with Duchenne muscular dystrophy.



Peter Mikkelsen, a 34-year old patient with Duchenne Muscular Dystrophy who attended the workshop said:

"The role for me as a patient in an ENMC workshop is to illustrate real-life experiences and describe short-comings of current care, particularly on the social issues we have to cope with".

"It was great to experience that all the medical professionals were proactively listening to us and incorporating our priorities and recommendations in the guidelines".

Testimonials



"SMA Europe feels the momentum for SMA has come and the whole SMA community works hard to seize it"

"The ventilation mask is troublesome as it comes loose during the night. Can we develop tailor-made masks and avoid the fear associated with night-time breathing?"

"Excessive daytime sleepiness and fatigue heavily impinge on the lives of DM-1 patients and their families."

"Now I am assured there are many committed professionals working hard to myotonic dystrophy patients, such as my wife"



Young Scientist Programme in 2014

This programme was set up in partnership with Genzyme in 2014 to give young researchers and clinicians in the neuromuscular field the opportunity to network and receive state-of-the-art scientific information. It was also aimed at raising awareness of the ENMC amongst young professionals.

Facts & Figures

In 2014 we invited for the first time young scientists to submit an application for attending a workshop within their field of interest and made three awards.

We would like to invite you to read the interview published on our website (www.enmc.org) with the young Canadian psychologist Dr. Benjamin Gallais. He works in a rehabilitation centre and specializes on myotonic dystrophy. He took part in the 207th ENMC workshop titled 'Respiratory Insufficiency in Patients with Myotonic Dystrophy' and presented the results of his PhD

"The ENMC application for a young scientist award is clear and the questions are meaningful: the relevance for my future research and creating new collaborations, getting myself recognised is actually the point I wanted to reach at the workshop as a young scientist".

Benjamin Gallais:

"The value of ENMC in organising workshops to me is that I could be personally involved in the workshop, the conclusions and the publications".



Benjamin Gallais, on the left, was the first one to receive an ENMC Young Scientist Award.

Increased ENMC visibility in 2014

As indicated by the Chair of the Executive Committee, the ENMC has started to focus on raising its profile within the scientific community. We were keen to be represented at international conferences so we can reach out to the new generation of young researchers and clinicians that would like to develop their expertise in the field of NMD. We anticipate this to be the new generation of workshop applicants and organisers and those that will continue the work on established consortia and even may establish new ones.

International conferences

This year the ENMC was represented in many conferences. Amongst them was the International Congress on Neuromuscular Diseases (ICNMD), the World Muscle Society meeting in Berlin (WMS) and the Dutch-Belgium-German Study Meeting on neuromuscular disorders. We also took part in the Conference of the International Society of Quality of Life Research (ISOQOL) to introduce the successful concept of the ENMC.

Professor Baziel van Engelen chaired the first international ENMC symposium at the ICNMD conference to discuss the **value of ENMC workshops**. The aim of this symposium was to raise awareness of the impact of the ENMC to establish a network of researchers and clinicians and to accelerate the development of treatments for people affected by a neuromuscular conditions.



The ENMC Research Director Professor George Padberg and the Vice Chair of the Executive Committee Dr. Raffaella Willmann as ENMC ambassadors at the ENMC booth at the WMS in Berlin.

ENMC and social media

Just recently the ENMC started to increasingly use social media to raise awareness of the ENMC amongst our stakeholders. During the last year we doubled our followers on Twitter and LinkedIn. Using social media we are able to disseminate the outcomes of the workshops faster and share news with the NMD community. Please follow, like and retweet us so we can broaden our network in 2015 as well!

WE DOUBLED OUR FOLLOWERS IN 2014



Visitors to the website

Our website does not only include an archive of past ENMC workshops and lay reports, it is also the place to find guidelines to apply for workshops, the young scientist programme, scientific publications and information about our three main programmes. In 2014, the ENMC website was visited on a regular basis. Of the 1,866 users, approximately 80% were new visitors and 20% were returning visitors. The visits came mainly from Europe but also from USA, Asia and Canada, showing the global interest in ENMC activities and achievements.

"OVER 1.000 VISITS PER MONTH"

Resources and Financial Management in 2014

Annual accounts for the year 2014 have been compiled in accordance with accounting principles generally accepted in the Netherlands. The financial accounts are drawn up in Euros. For a brief summary see the balance sheet in the table below.

Financial Summary

Balance Sheet Summary as of 31 December 2014 (in Euros)		
CURRENT ASSETS	31-12-2014	31-12-2013
Other receivables	32,750	46,573
Cash at banks	427,031	375,038
Totals	459,781	421,611
EQUITY AND LIABILITIES		
Equity	424,941	354,555
Current liabilities	34,840	67,056
Totals	459,781	421,611

In the table below, the overall income and expenses have been summarized over the year 2014, in comparison with the budget for 2014 and the actual figures for the financial year 2013. The difference in budget and actual figures for 2014 (€ 87,386) has resulted for different reasons.

- Only six ENMC applications were selected for workshops in 2014, while eight were budgeted (€ 39,599)
- More income was generated from other (workshop-specific sponsoring) contribution than anticipated (€ 19,190)
- Personnel expenses were lower (€ 28,660) due to a lower annual salary for the current Managing Director, who was appointed mid-February 2014.

Financial Statement income and expenses

Statement of income and expenses for the year 2014			
	2014 (€)	Budget 2014 (€)	2013 (€)
Contribution			
Full Members	210,000	210,000	210,000
Associated Members	5,000	5,000	5,000
Other	88,690	68,000	47,571
Total Income	303,690	283,000	262,571
Expenses			
Personnel Expenses	104,920	134,000	116,316
Housing Expenses	11,000	12,000	11,000
Workshop Activity Expenses	80,401	120,000	93,616
Organisational Expenses	40,732	40,500	41,961
Total Expenses	237,053	306,500	262,893
Operating Result	66,637	-23,500	-322
Interest Income	3,749	5,000	4,764
Net Result	70,386	-18,500	4,442

Opinion of the auditors

The ENMC accountants have verified and approved the annual accounts. For downloading the full PDF version of the annual accounts report of 2014, we refer to the ENMC website.

Acknowledgements

Thanks to the continuous support of the nine European patient organizations, with representatives in our Executive Committee, the ENMC is able to facilitate and organise on average seven workshops per year. With the support from additional partner organizations, such as condition-specific associations and pharmaceutical companies, we are also able to invite participants from non-ENMC countries.

ENMC full and associated members:



Finnish Neuromuscular Disorders Association



Deutsche Gesellschaft für Muskelkranke e.V. DGM



MUSKELSVINDFONDEN



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



Workshop-specific sponsors:



ZonMw



SMA
FOUNDATION



Parent Project
Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE



GlaxoSmithKline
Rare Diseases

genzyme
A SANOFI COMPANY

BiOMARIN
BioMarin Europe Ltd.

MDA[®] Muscular
Dystrophy
Association
Fighting Muscle Disease

Looking Forward to 2015

In the coming year, 2015, we continue to be dedicated to our **ENMC activities**: Five workshops are being scheduled for 2015 and seven applications have been submitted for review in the spring of 2015. If they are selected for an ENMC workshop, they will be planned for the second half of 2015 and the first quarter of 2016.

We would like to expand the Young Scientist Programme and we will continue our efforts to encourage patient participation in the ENMC workshops. We have implemented an additional initiative to facilitate these programmes, the ENMC Company Forum. This is a group of pharmaceutical and biotechnology companies together helping to ensure sustainability of ENMC activities.

Finance

Our aim is to increase our budget by 20% through workshop-specific donations and memberships to the Company Forum in order to meet the increased demand for ENMC workshops.

Notable upcoming events

The ENMC anticipates organising nine high-quality workshops in 2015. We will continue to provide support to our network helping researchers, clinicians and lay people in various activities. With regard to international conferences, the ENMC will be represented at the WMS meeting in Brighton, UK, which will take place from 30 September to 4 October 2015.



European Neuromuscular Centre (ENMC)
Lt. Gen. van Heutszlaan 6
3743 JN Baarn
The Netherlands

+31 35 5480481
enmc@enmc.org
www.enmc.org