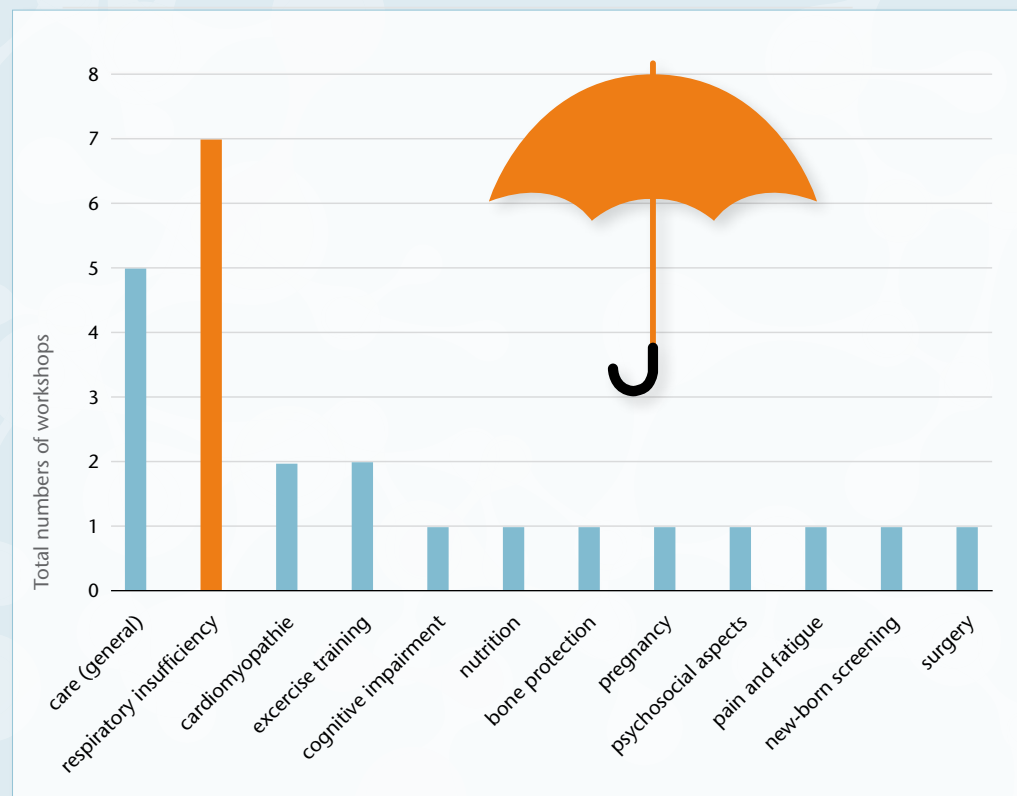


ENMC How can ENMC workshops encourage exchange of successful outcomes from one disorder to another?

DR. MICHEL TOUSSAINT ENMC workshops are the ideal platform to enhance collaboration with experts who work on different disease types. During our discussions, we found it was key to compare the impact of airway clearance techniques in different disorders and in different age groups. We needed expert opinion regarding implementation of each technique in the treatment of infants, children, adolescents and adults; with or without tracheostomy; with or without assisted ventilation; with or without scoliosis; with or without bulbar weakness; and with or without the ability to cooperate physically. ●

Medical care and patient-oriented topics at ENMC “Umbrella” workshops in 25 years’ time^{1,2}



Michel Toussaint:
“The topic of our workshop – ventilation – is applicable across many neuromuscular disorders, and as such it is an example of ENMC’s ‘umbrella’ workshops.”

¹ Breukel et al. The impact of European Neuromuscular Centre (ENMC) workshops on the neuromuscular field; 25 years on. Neuromuscular Disorders (April 2019) Volume 29, Issue 4, Pages 330–340.

² Van Wijk et al. Bibliometric research performance of the European Neuromuscular Centre (ENMC) 2000 – 2016/1. www.enmc.org (October 2018).

A special thanks to all our members and other supporters

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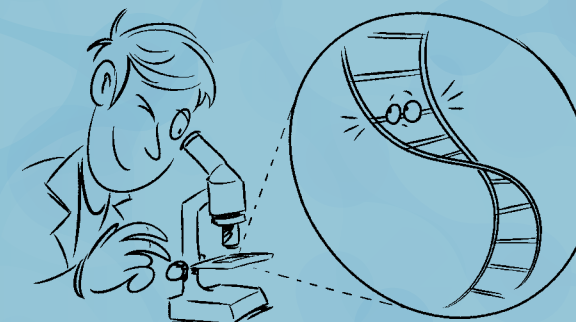
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ENMC

Connecting healthcare professionals worldwide in order to advance progress in the diagnosis, treatment and care of people with a neuromuscular condition.

Impact for the neuromuscular community and for patients

From bench ...



... to bedside!



The European Neuromuscular Centre (ENMC)

has organised more than 250 international workshops to bring together experts in the field of neuromuscular disorders (NMD); patients, researchers, clinicians and healthcare professionals. Patients affected by a neuromuscular condition and their relatives have participated in these workshops to bring in the patient’s voice.

Whether these workshops indeed resulted in research collaborations and improvements for patients, was evaluated two years ago.^{1,2} This leaflet highlights the outcomes of the analysis and reports the experience of two workshop organisers: Prof. Mary Reilly and Dr. Michel Toussaint.



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ENMC workshops

ENMC workshops are often a starting point for consortia that use the platform successfully to sustain their collaborations over time and to write new research proposals, for instance for European grant calls. Over the last 25 years, the topics of workshops have gradually moved from basic science and clinical themes to more patient-related topics.

This is well illustrated by the Charcot-Marie-Tooth (CMT) consortium, which held nine consecutive ENMC workshops in a period of 20 years.



Dr Michel Toussaint is a physiotherapist working at the Centre for Home Mechanical Ventilation, Department of Rehabilitation at the Inkendaal Hospital in Belgium.

Michel Toussaint, organised and participated in many workshops on the topic of ventilation in patients with neuromuscular conditions. He discusses the outcomes of workshops focusing on ventilation and the significance of collaboration across disorders.

INTERVIEW PROF. MARY REILLY

ENMC Prof. Reilly, how do ENMC workshops help patients?

PROF. MARY REILLY Back in 1997 the first two workshops were held on the original classification and diagnosis of CMT. These allowed the careful phenotyping of patients so that families could be combined for gene identification. Subsequent workshops allowed the CMT research community to collectively agree what outcome measures to use in clinical trials for CMT.

Later, studies had indicated that ascorbic acid could be beneficial in the treatment of this disease and all of the significant Vitamin C trials in the mid-2000s used the CMT neuropathy score (CMTNS). This primary outcome measure was selected as part of a trial protocol which was developed at an ENMC workshop. Analysis from these trials suggested that Vitamin C in fact did not work for Charcot-Marie-Tooth Disease Type 1A, and this directly impacted patient care.

Our most recent CMT workshop has also led to direct improvements in patient care; e.g. considering surgery for foot deformities in patients with CMT, we agreed to avoid certain surgery treatment modalities. Furthermore, we reached consensus on critical future research questions.

ENMC How does ENMC collaboration help the research community?

PROF. MARY REILLY The ENMC has helped unite the CMT research community and this has led to a new special interest group within the international peripheral nerve society called 'the CMTR' (CMT and related disorders) which is truly international. ●

RATE OF
ACHIEVEMENT
~ 90%^{1,2}

Mary Reilly is Professor of Clinical Neurology and Consultant Neurologist at the National Hospital for Neurology and Neurosurgery, London and an important driving force of the CMT consortium. Here Mary talks about the invaluable role played by ENMC workshops in furthering research into CMT Disease.

"Achieving such a high percentage of our workshop deliverables has brought knowledge and new collaborations to the research field, improving designs of clinical (investigator and pharma-driven) trials, and innovating diagnostic tools for the patients."



INTERVIEW DR. MICHEL TOUSSAINT

ENMC Dr Toussaint, what direct outcomes that change clinical practice can emerge from ENMC workshops?

DR. MICHEL TOUSSAINT Our most recent ventilation workshop, in 2017, focused on airway clearance techniques in several neuromuscular disorders (NMDs) and led directly to the production of important and comprehensive guidelines on airway clearance in NMDs. These guidelines will be the international reference for the next 10 years.

One example of a workshop outcome which had direct impact on the respiratory care of NMD patients.

Michel continues: Our recommendations are universal and can be applied irrespective of financial resources, which of course differ from country to country. Instrumental techniques of airway clearance are not always costly and we were able to illustrate that successful airway clearance is more dependent on knowledge than on budget.

ENMC How do ENMC workshops encourage collaboration?

DR. MICHEL TOUSSAINT ENMC workshops provide a comfortable atmosphere where experts and individuals with lived experience of NMDs can meet for a three-day, closed-door meeting, and have time to enter deep discussions. In this environment, we were able to achieve a remarkable level of agreement about our guidelines' final text, figures and algorithms and we are particularly proud about this.

Different members of our group have disseminated the outcomes of the workshop via international meetings on respiratory care, and we know that our recommendations are being widely used and highly cited in scientific papers.

→ continues on the next page

CITATION
SCORE
= 124%^{1,2}

"THE NEXT ENMC WORKSHOP
ON THIS TOPIC WAS ALREADY
SET FOR 2020."