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"The impact of European Neuromuscular Centre (ENMC) workshops on the neuromuscular field; 25 years on …"

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Abstract

Since 1992, the European Neuromuscular Centre facilitated workshops to bring experts in the field of neuromuscular disorders together. After organising more than 235 workshops, it is time to evaluate what impact these 25 years of ENMC workshops have had on the neuromuscular research field and on people affected by a neuromuscular condition. To measure this, workshop topics were retrospectively evaluated and bibliometric analyses on the citation scores of ENMC-derived publications were performed. In addition, a personalized survey was used to investigate the actual achievement and implementation of workshop deliverables. The evaluation of 25 years' workshop topics revealed a strong representation of muscular dystrophies, congenital and mitochondrial myopathies. The publications derived from ENMC workshops scored "high impact" as illustrated by the Mean Normalized Citation Score of 1.24. Also 16% of the ENMC papers belong to the top 10% best cited articles in the neuromuscular field. The main outcome of the personalised survey was that 90% of all workshop deliverables were started and either ongoing or completed. Of these deliverables, 78% were implemented in the field; bringing state-of-the-art knowledge and new collaborations to researchers and clinicians, improving designs of clinical trials and innovating tools to make accurate diagnoses.

Keywords: Impact; ENMC workshops; Bibliometric analysis; Survey; Citations.

1. Introduction

The ENMC was founded in 1992 by a group of European patient associations with the aim of bringing together leading researchers and clinicians with expertise in neuromuscular science from all over the world [1]. Prof. Alan Emery and other founding fathers are greatly acknowledged for their tremendous input in the first 10 years of the ENMC. The mission of the ENMC is to encourage and facilitate communication and collaboration in the field of neuromuscular research with the aim of improving diagnosis and prognosis, finding effective treatments and optimizing standards of care to improve the quality of life of people affected by neuromuscular disorders (NMD). The ENMC achieves this mission by financing and organizing workshops on topics that vary from finding the genetic cause for neuromuscular conditions, designing clinical trials to test new drugs and improving care for neuromuscular patients, i.e. the full translational range [2]. The ENMC informs scientists on the outcomes of each single workshop via publications in acknowledged journals and the lay community via short reports, its website and social media. With 237 workshops by September 2018, the ENMC established a network of over 2500 researchers, clinicians and patients from approximately 65 countries, creating international cross-talk and world-wide collaboration in basic research and clinical trials. The workshop applications are peer-reviewed bi-annually by the ENMC Research Committee members who judge the scientific quality, relevance, timing and participants of workshop applications. They advise the Executive Committee, which is an independent board of representatives of patient

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organisations governing the ENMC and deciding on approval of workshops.

Aim of this project was to assess the impact of ENMC workshops as scientific output to the research and patient community, in order to measure its own performance and review the realization of its mission.

One dimension of impact can be assessed on the basis of the publication output and the citation scores that mirror on one side the degree of scientific dissemination of the results obtained in a workshop, on the other side the extent of scientific collaborations emerging from the workshops. For this analysis we made use of the Elsevier Database of Scopus, since all workshop reports were published in Neuromuscular Disorders. In addition, our publication output was compared with relevant neuromuscular literature using Web of Science cluster normalization.

In addition, the ENMC wanted to investigate to what extent ENMC workshops have led to dissemination and implementation in the society, for instance by resulting in direct improvements for patients and their families at the level of diagnosis and prognosis, effective treatments and standards of care. To answer these questions, an evaluation of 25 years' workshop topics and a more detailed survey on workshop deliverables in a 5-year cohort was performed.

2. Methods

2.1. Bibliometric analysis of ENMC workshop reports and publications derived from ENMC workshops

2.1.1. ENMC workshop reports – publication years 1993–2016 (Scopus)

For all individual ENMC workshop reports in this period the Scopus citation score was assessed.

- Source of database: https://www.nmd-journal.com
- Period: 1993–2016
- Keywords used: the number of each ENMC workshop report within this period (Xxth workshop)
- Number of output: 157 ENMC workshop reports

2.1.2. Search-results and literature normalization – publication years 2000–2016 (WoS)

This analysis was performed by the Centre for Science and Technology (CWTS), in Leiden, The Netherlands. The full CWTS report is published on the ENMC website (www.enmc.org).

- Source of database: CWTS in-house version of the WoS Citation Index (CI) database.
- Period: 2000–2016 (this period is shorter than with the Scopus assessment, since the CWTS-CI database only contains normalization tools from 2000 onwards)
- Keywords used: 'ENMC' or 'EUR* NEUROMUSC* CTR' or 'EUR* NEUROMUSC* CENTER' or 'EUR* NEUROMUSC* CENTRE' within all searchable data fields in the CI.
- Document types: 'article', 'letter' and 'review'
- Number of output: 98 ENMC workshop derived publications

2.1.3. Internal coverage of publications within the CI database

As a rule, whenever internal coverage drops below 50%, the non-CI citation environment is as important as the environment within the CI used for analysis. The internal coverage for the ENMC network in the year 2000–2016 is 88%, which indicates that the CI is an appropriate tool to perform the ENMC analyses with confidence and results will be robust with meaningful indicators.

2.1.4. Normalized indicators of citation impact

To account for age and field differences in citations, CWTS uses normalized citation indicators. The main normalized indicator for impact of citations is the MNCS, the mean normalized citation score. This parameter can be calculated as the average number of citations of a set of papers, normalized for the year of publication and scientific field citation difference [3,4].

2.1.5. Collaboration profile

In the collaboration analyses, CWTS casts the output (% share in the total number of papers (P) and MNCS) of three different types of collaboration against their input: "No Collaboration" (only one single institute address), "National collaboration" (only addresses originating from one country) and "International collaboration" (more than one country affiliated in the addresses).

2.2. Impact analysis of ENMC workshops

2.2.1. A retrospective evaluation of the topics of workshops (1992 – 2017)

We categorized the topics using the archive of all workshop titles since 1992 according to the disease classifications used by Prinses Beatrix Fonds and Muscular Dystrophy UK.

General (or horizontal) workshops are defined as workshops that discuss common issues for multiple neuromuscular disorders (NMDs), instead of focussing on a specific disorder. Care workshops cover topics which are close to the patients' needs and discuss solutions for the daily management of the disease. These workshops focus on either one specific NMD class or on multiple NMDs.

2.2.2. A personalized survey in a cohort of 38 workshops (2010–2014)

The 5 year period (2010–2014) was selected taking into account a lag time of 2 years for completion of the deliverables. Workshop organisers, researchers, clinicians and patient representatives who participated in the workshop were randomly selected for this survey. The aim was to have at least 2–3 respondents for each workshop in the survey, so that multiple perspectives were included in the analysis. All workshop deliverables ($n = 202$) in this cohort were identified upfront from the original applications and full reports. On

Table 1
Overview of the bibliometric analysis by CWTS. The main impact and visibility indicators are the MNCS and the PPtop10%, respectively, and these were chosen as the key indicators in this study.

Indicator	Dimension	Definition
P	Output	Total number of publications.
TCS	Impact	Total number of citations.
MCS	Impact	Average number of citations.
TNCS	Impact	Total normalized number of citations.
MNCS	Impact	Average normalized number of citations.
PPtop10%	Impact	Proportion of publications that belong to the top 10% of their field. The "visibility"-index as highly cited work tends to be noted more. (PPtop1% is therefore the percentage share in the top 1% cited publications).
PPnC	Impact	Proportion of uncited publications.
MNJS	Journal impact	Average normalized citation impact of a journal.
No Collaboration	Collaboration	Proportion of publications authored by a single institution.
National Collaboration	Collaboration	Proportion of publications resulted from national collaboration.
International Collaboration	Collaboration	Proportion of publications resulted from international collaboration.

average 4–5 deliverables per workshop could be identified. The identified deliverables were categorized according to the definitions summarized in Table 2. This categorization revealed a concise distribution of research topics that were addressed in the workshops of the 5-year cohort. In the survey it was asked whether specific workshop deliverables were "completed", "started and ongoing", "started with delay", or "never started"; and in addition, whether they were "implemented" in the NMD field (or not) with source references. If deliverables were never worked out at or after a workshop, respondents were asked to tick the reasons: "lack of resources", "lack of finances", "lack of personnel", "lack of commitment", "lack of consensus due to various reasons" and/or "other". The survey was successfully piloted with approximately 10 participants and subsequently rolled out for 38 workshops. We received a response for 29 out of 38 workshops. From the 202 deliverables identified upfront, we received data on 153 deliverables (76%). These 153 deliverables have been analyzed and results are presented in this paper. In the 5-year cohort, some deliverables were categorized as "Consortia/networks" or "Collaborative research groups".

Discrimination was made between *achieving* deliverables at or directly after the workshop and *implementing* these deliverables in the neuromuscular field, in order to bring a workshop output directly to the patient. The "deliverables achievement status" could be scored according to Fig. 1.

Table 2
Definition of the deliverable categories in the 5-year cohort. The deliverables of the specific workshops in the survey were mentioned in the application and full NMD report. They were categorized according to the definitions stated in this Table.

Deliverable category	Definitions of categories
Guidelines care	International standards for care and therapy management, excluding drug treatments
Guidelines diagnosis	International standards for diagnosis
Guidelines therapy	International standards for drug interventions
Other guidelines	International standards for guidelines other than mentioned above; such as animal model experimentation, translational research, outcome measures
Evaluation study	Evaluation of running studies, of data collection, discussion of results, progress of training
Clinical trial or natural history study setup	Defining outcomes, setting up design of a (clinical) study, trial readiness
Database	Launch of a database or sample repository
Registry	Collection of standardized patient data, medical history, outcomes of interventions
Teaching	Course, student exchange
Scientific overview /summary	Sharing/update of state-of-the-art knowledge
Collaborative research	Setting up of collaborations also with funding agencies or regulatory agencies, working groups for a clinical trial, exchange of data and bio-samples, informal partnership/loose consortium or network
Consortia, networks	Structured form of partnership with a well-defined structured governance arrangement (steering board, rules, meeting infrastructure)
Other	Any other category

3. Results

3.1. Results of the bibliometric analysis of ENMC-workshop derived publications

3.1.1. Patients and families

Informing patients and their families about the achievements of ENMC workshops is one of the key priorities of the ENMC. This is done by the *workshop lay report*, which is written by workshop participants and published on the ENMC website (www.enmc.org) within two weeks time after the workshop.

In the online ENMC archive (set-up since 2000) of lay reports only one workshop was missing, resulting in a 99% publication rate of lay reports. Nowadays, all lay reports are being translated in many different languages other than English, which increases the accessibility of these reports for people worldwide. The nine European patient member organisations help to disseminate these translated lay reports via their local patient networks. Furthermore, the ENMC creates awareness on social media whenever lay reports have been published at the website.

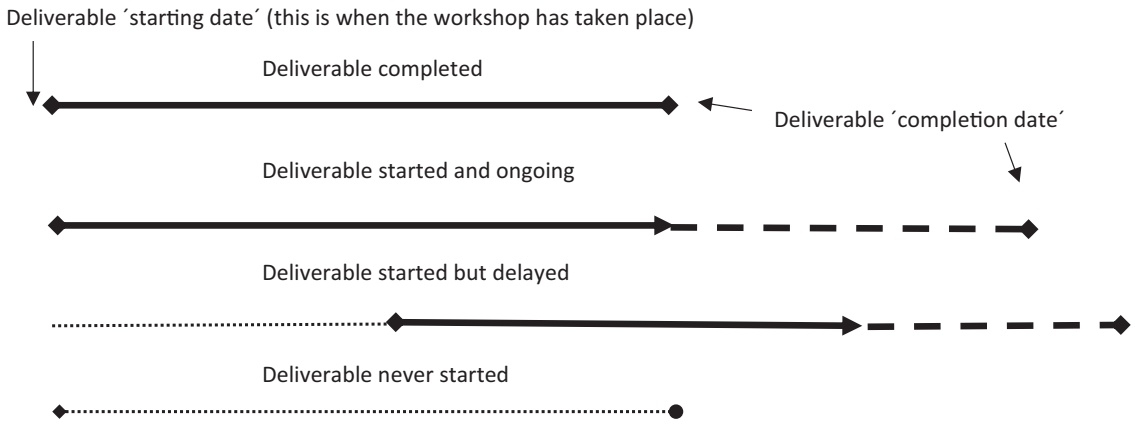

Fig. 1. Definition of deliverable achievement status: this scheme was used to collect data on the progress of the workshop deliverables. Questions and answers in the survey were standardized and survey respondents could tick the boxes electronically. If needed, there was an open comment section, which generated narratives to support the quantitative data.

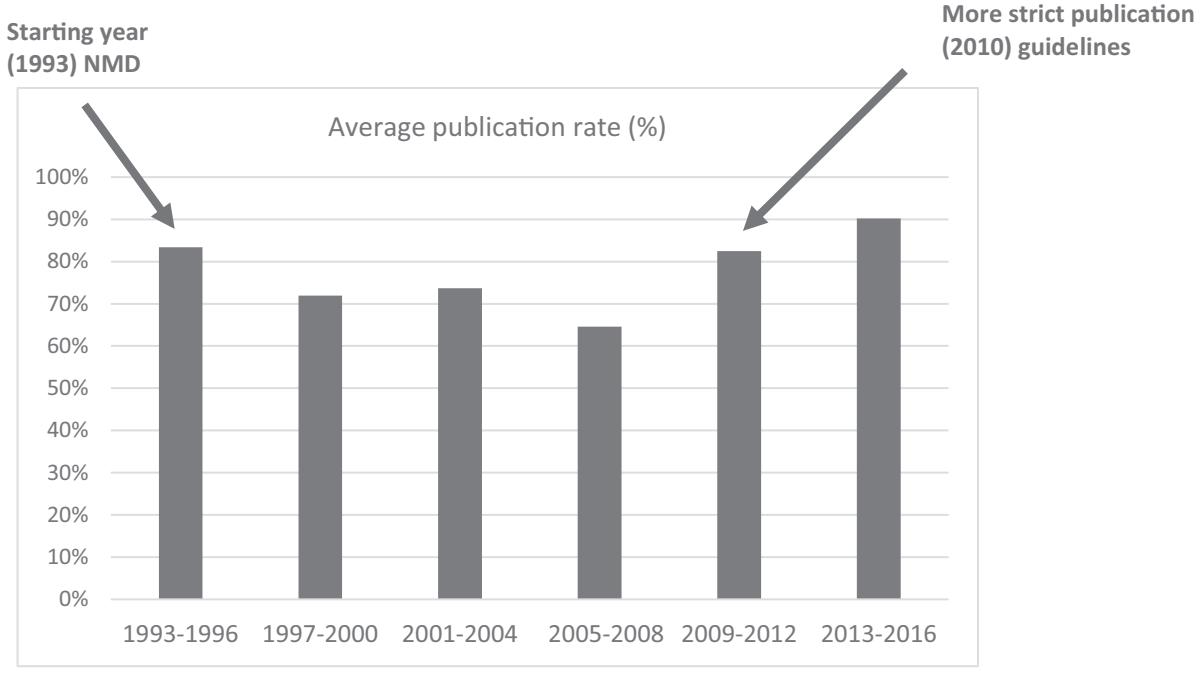

Fig. 2. Percentage of full publications reporting on ENMC workshops in the last 25 years. For each ENMC workshop that took place in the period 1992–2016, it was assessed whether it resulted in a full report in Neuromuscular Disorders. This was expressed as the annual publication rate (in%) and was averaged over periods of 4 years to show possible trends.

3.1.2. Research community

Researchers, clinicians, and health care providers who are active in the field of rare neuromuscular disorders need to be able to read about the scientific results of ENMC workshops in the literature. It is mandatory that workshop organisers submit a *full workshop report* to Neuromuscular Disorders within 6 months' time after the workshop has taken place. Over the 25 year period, on average 79% of the workshops were published as full reports in Neuromuscular Disorders. Due to more strict reporting guidelines, which became effective in 2010, this percentage has increased to 90% since then (see Fig. 2).

ENMC workshop reports – publication years 1993–2016 (Scopus): The number of times ENMC workshop reports were cited is shown in Fig. 3. The majority of ENMC workshop reports appear to be cited in the range of 0–50 times. Nevertheless, many individual reports were cited more than 50 times. The four workshop reports with the highest citations, but not included in Fig. 3, were the publications of Workshop #124 on the "Gold standard for Duchenne Muscular Dystrophy therapy" (129 citations) [5]; Workshop #30/31 on "Limb Girdle Muscular Dystrophy nomenclature" (156 citations) [6]; Workshop #107 on "Cardiac involvement in NMD" (212 citations) [7] and Workshop #119 on "Trial

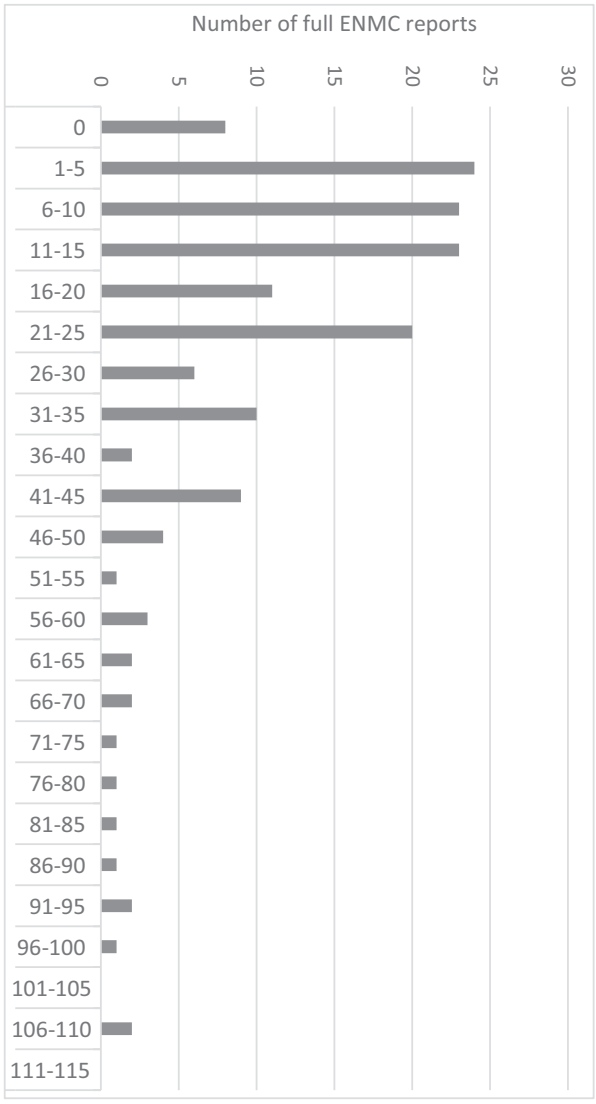

Fig. 3. Absolute citation score of the ENMC workshop reports (1993–2016). Data derived from Scopus (Neuromuscular Disorders, Elsevier) as reported in June 2018.

design in Idiopathic Inflammatory Myopathy" (390 citations) [8].

Limitations of this evaluation are that (i) the Scopus citations scores are *absolute* values and hence are not correcting for differences in publication age (how many years a paper is available for citations) and cultural citation behavior per scientific area. Therefore, they cannot be compared with citations scores of other papers; (ii) the output of ENMC workshops is not only reflected by the mandatory ENMC workshop reports, but also by the spontaneous publications reflecting and/or referring to (collaborative) work started at an ENMC workshop. To bypass these limitations, a literature search on ENMC-workshop derived papers within the CWTS in-house CI database was performed and on the resulting list of papers, *relative* citation scores were identified by using WoS cluster normalization to correct for year and field differences (see next section).

Search-results and literature normalization – publication years 2000–2016 (in-house CI database WoS): The initial data selection yielded a validated dataset of 98 papers, of which approximately 30% were ENMC workshop reports and 70% indirect publications. Apparently not every Neuromuscular Disorders year was included in the in-house CI (WoS) database or not every publication identified as review or article, which explains the smaller amount of ENMC reports in this selection. Nevertheless, the available data set was robust enough to allow the analysis as confirmed by the high internal coverage rate of 88%. Since the impact of the ENMC workshops not only entails the full workshop reports, but also follow-up research and collaboration activities after the workshop, citations scores of all 98 papers were measured. Hence, this analysis reflects performance of the ENMC network, not of the ENMC as a "sole institute". This set of papers is referred to as "ENMC-workshop derived publications".

The output (P) trend started to increase around 2010, when the full report publications became mandatory (see Table 3). The total number of citations (TCS) is 1955 with a Mean Citation Score (MCS) of 19,95. The impact score of the ENMC-workshop derived papers, normalized for literature within the related clusters is 1.24 (MNCS); meaning that the impact of this selection of publications is 24% higher than the impact of the average performing paper (MNCS of 1) in this cluster of the CI database. The threshold, at which CWTS assigns the 'High Impact' label, is an MNCS of 1.20; exceeding by 20% the world average. The proportion of papers that belong to the top 10% of their field (PP Top 10%) is 16% which means that ENMC-workshop derived papers have a 6% higher visibility than world average. In addition, the PP Top 10% representation is in most 4-year periods in line and evolves much in accordance with the direction of the MNCS, supporting the robustness of the results.

Collaboration profile: Since the ENMC aims to encourage and facilitate communication and collaboration in the field of neuromuscular research, CWTS performed a collaboration analysis to check for the effectiveness of ENMC in achieving this part of its mission. In the collaboration analysis a distinction is made between papers published by individual researchers/institutes ('no collaboration'), by research groups in one single country ('national collaboration') and by research institutes across international borders ('international collaboration') (see Fig. 4).

Publications authored by single institutes achieved the highest MNCS (1,41), but had the lowest share of the output (17%). Publications authored by international collaborations had the highest share of ENMC workshop derived papers ($n = 55$ papers, 56%); of these, 32 were workshop reports. Overall, this set of papers published by international collaborative groups had a high impact citation score (MNCS = 1,28). Publications authored by national collaborations were cited at world average and made up approximately 25% of all ENMC workshop derived papers in this analysis.

Table 3
Impact ENMC-workshop derived publications (2000 – 2016). Citations were measured by WoS publication cluster normalization and were corrected for self-citations. Abbreviations: see Table 1.

Year	P	MCS	TCS	MNCS	MNJS	TNCS	PP top 10%	PP uncited	Self citations	PP collab	PP int collab
2000 - 2016	98	19.95	1955.00	1.24	1.13	121.85	16%	6%	20%	83%	56%
2000 - 2003	16	7.81	125.00	0.88	0.89	14.01	6%	13%	31%	81%	44%
2001 - 2004	13	12.08	157.00	1.39	1.23	18.03	15%	15%	31%	85%	54%
2002 - 2005	11	5.82	64.00	1.02	0.68	11.20	9%	9%	32%	82%	55%
2003 - 2006	11	7.09	78.00	1.02	0.73	11.20	9%	9%	20%	73%	45%
2004 - 2007	10	11.10	111.00	1.21	0.82	12.08	10%	0%	19%	70%	60%
2005 - 2008	9	8.00	72.00	1.90	1.28	17.08	22%	0%	11%	67%	33%
2006 - 2009	10	11.40	114.00	1.54	0.96	15.43	24%	10%	11%	80%	40%
2007 - 2010	19	6.37	121.00	1.22	1.09	23.14	16%	16%	21%	84%	47%
2008 - 2011	20	8.85	177.00	1.25	1.14	25.07	15%	0%	22%	75%	40%
2009 - 2012	19	6.58	125.00	0.95	1.02	18.11	5%	0%	29%	79%	47%
2010 - 2013	35	6.11	214.00	1.24	1.21	43.38	10%	11%	25%	77%	51%
2011 - 2014	30	6.57	197.00	1.43	1.33	42.87	23%	7%	18%	80%	60%
2012 - 2015	43	7.56	325.00	1.25	1.22	53.92	14%	14%	20%	86%	63%
2013 - 2016	50	9.26	463.00	1.27	1.20	63.68	17%	12%	23%	88%	66%

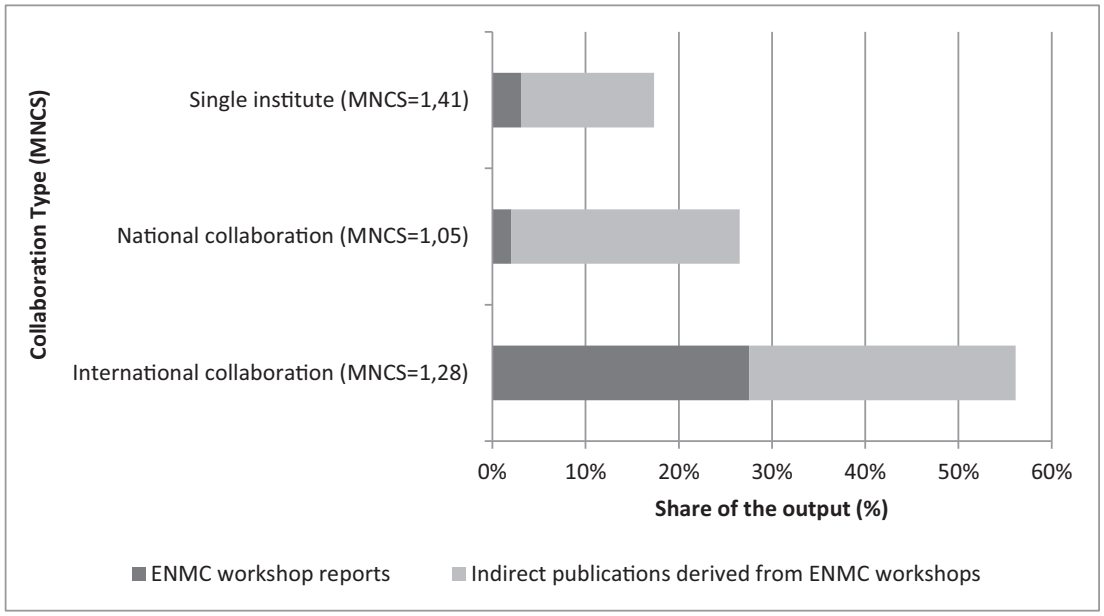

Fig. 4. Collaboration profile for the ENMC-workshop derived publications (2000–2016). In this figure, the share of the output, in% of the total number of publications within the period 2000–2016 fulfilling ENMC search criteria, is drawn on the X-axis. The type of collaboration is drawn on the Y-axis with the impact (MNCS) of each group in brackets. The ENMC workshop reports are illustrated in dark grey bars, the indirect publications in light grey.

Further analysis provides more insight in nodes of collaboration in this set of ENMC publications and how these core centers are connected to others which are more closely or less frequently connected, see CWTS report on www.enmc.org.

3.2. Impact analysis of ENMC workshops

In this part of the study, two analyses were performed: 1) a global retrospective evaluation of workshop titles in the past 25-years to get an overview of discussed disease areas and topics of research, in order to identify neglected workshop topics and 2) a detailed analysis of the level of achievement of workshop deliverables in a 5-year cohort using a personalized survey.

3.2.1. A retrospective evaluation of the topics of workshops (1992 – 2017)

Supported by the NMD full reports of workshops and the archive of lay reports, a full list of approximately 200 workshops taking place from 1992 to 2017 allowed to make an inventory how many times a certain disease class was discussed at an ENMC workshop. This revealed a strong representation of muscular dystrophies, congenital and mitochondrial myopathies (see Fig. 5), whereas myotonias and some ultra-rare conditions were topic of only one workshop during the last 25 years. It should be noted,

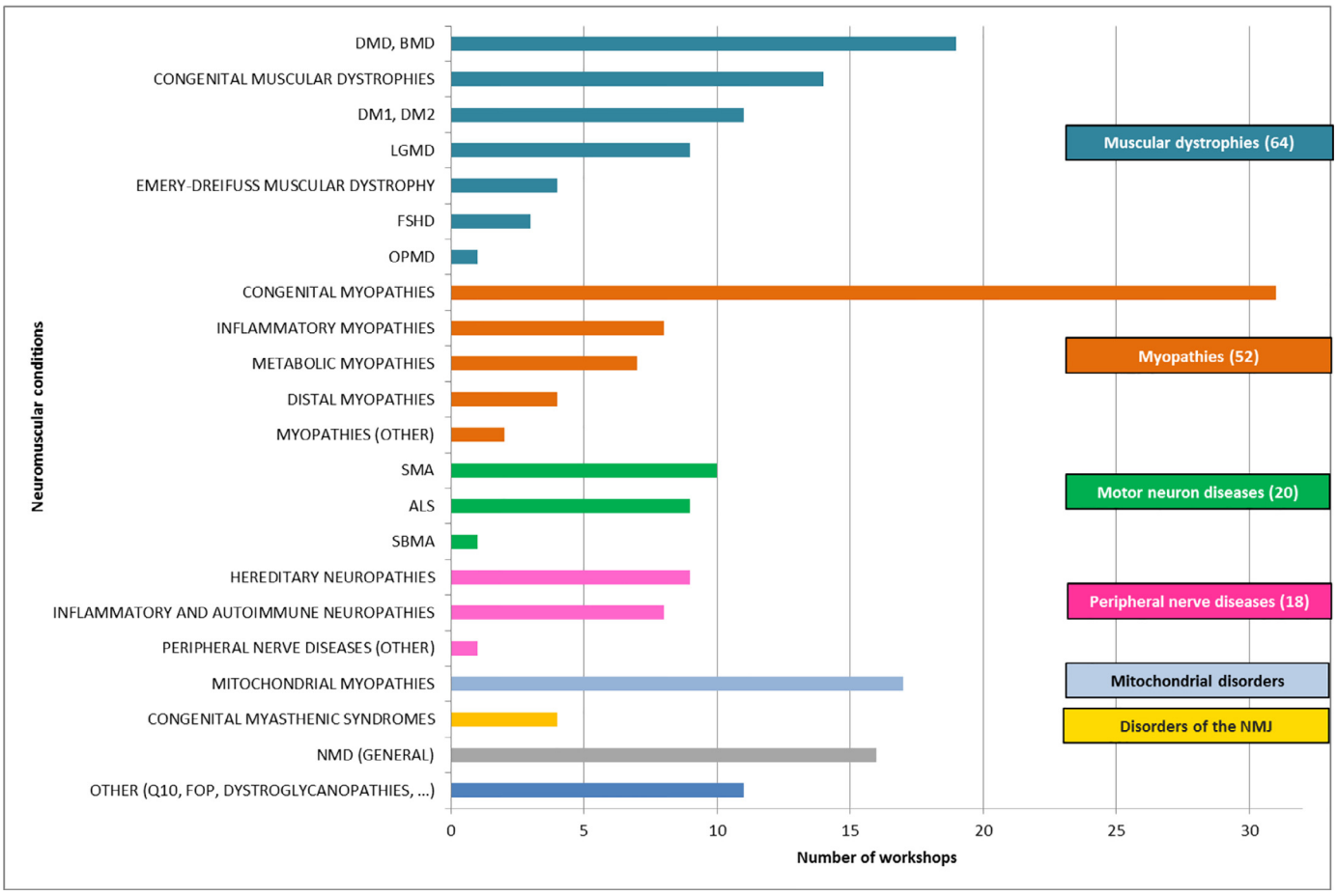

Fig. 5. Neuromuscular conditions topic of discussion at ENMC workshops (1992–2016). This figure illustrates the number of times a disease class was topic of an ENMC workshop in the last 25 years, e.g. muscular dystrophies were the most frequently discussed disease types (64 times) in 25 years with almost 20 workshops dedicated to Duchenne muscular dystrophy. This was closely followed by the myopathies with 52 workshops. If multiple neuromuscular diseases were covered in one workshop, this workshop was addressed as a "NMD General" workshop. In such a workshop, a common topic important for multiple NMDs was addressed, such as standards of care, ventilation or management of pain and fatigue. Abbreviations: ALS = amyotrophic lateral sclerosis; BMD = Becker muscular dystrophy; DM1 or DM2 = myotonic dystrophy type 1 or 2; DMD = Duchenne muscular dystrophy; FOP = fibrodysplasia (myositis) ossificans progressiva; LGMD = limb girdle muscular dystrophy; NMD = neuromuscular disorder; NMJ = neuromuscular junction; OPMD = oculopharyngeal muscular dystrophy; Q10 = co-enzym Q (ubiquinone)−10; SBMA = spinal bulbar muscular atrophy; SMA = spinal muscular atrophy. *Muscular Dystrophy UK and Prinses Beatrix Spierfonds the Netherlands are greatly thanked for their help with the NMD classification.*

though, that often these ultra-rare conditions were discussed in dedicated sessions of more general workshops.

Additionally, we looked at ENMC workshops covering care management in more detail ($n = 24$ ENMC workshops). These workshops focussed either on a specific neuromuscular condition (DMD, SMA, myotonic dystrophy, McArdle, ALS etc.) or on NMDs in general (see Fig. 5, gray bar). Topics like respiratory insufficiency and cardiac myopathy were most popular, whereas topics like pregnancy, pain and fatigue were addressed in only one workshop in the last 25 years (see Fig. 6). In 2018, the ENMC organised a special workshop entirely focussed on patient participation in research, care and quality of life issues and recently, several workshop applications with a care topic as focus of the workshop, e.g. cardiac and bone issues that NMD patients experience, were selected by the ENMC.

3.2.2. Level of achievement of workshop deliverables: bringing ENMC workshop deliverables directly to the patient (5-year survey results)

Since the retrospective evaluation of topics in 25 years did not provide detailed insight in the specific outcomes of ENMC workshops, a 5-year cohort was selected to investigate in-depth the deliverables and their implementation within the NMD research field. Scientific overview (knowledge sharing), starting collaborations (in the form of a network or consortium) and setting up clinical trials and natural history studies were most frequently aimed for deliverables in the 38 ENMC workshops (see Fig. 7). Sometimes deliverables were assigned to more than one category. Whereas diagnosis guidelines were deliverables in many workshops, therapy and care guideline deliverables were much less abundant. Set-up of (global) registries and databases were deliverables in 10% of the workshops. This may reflect the natural sequence of research within the field of NMDs, that started with

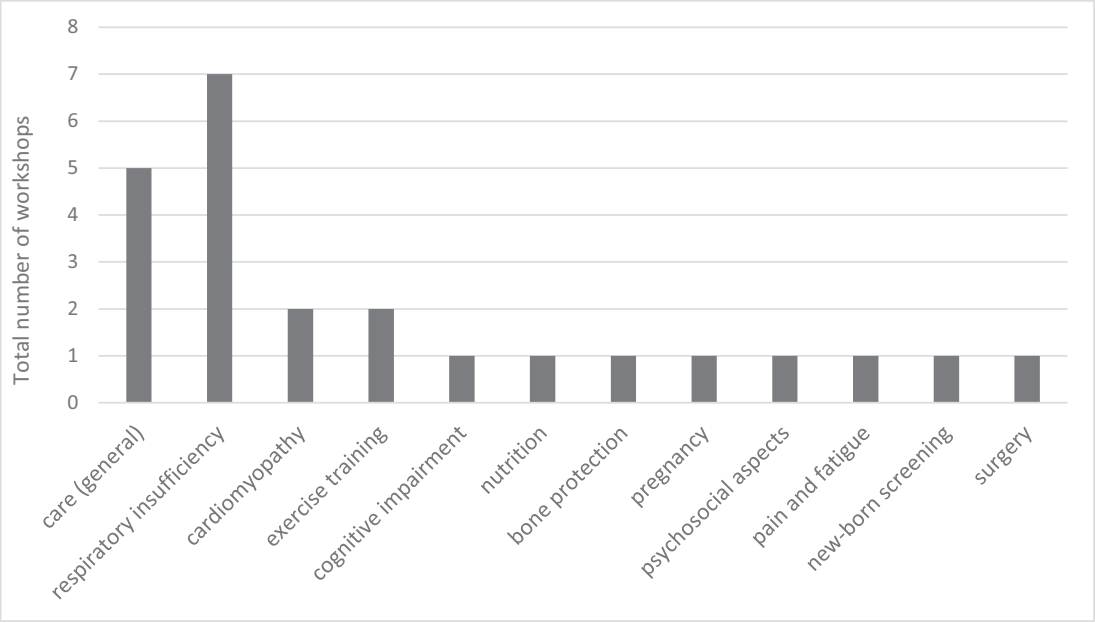

Fig. 6. Medical care and patient-oriented topics at ENMC workshops (1992–2016). In total, 24 ENMC workshops focussed on care management. Of these 24, 16 workshops dealt with common care topics across multiple neuromuscular conditions and 8 discussed care topics specific for individual NMDs (ALS, DMD, CMT, McArdle, SMA etc.).

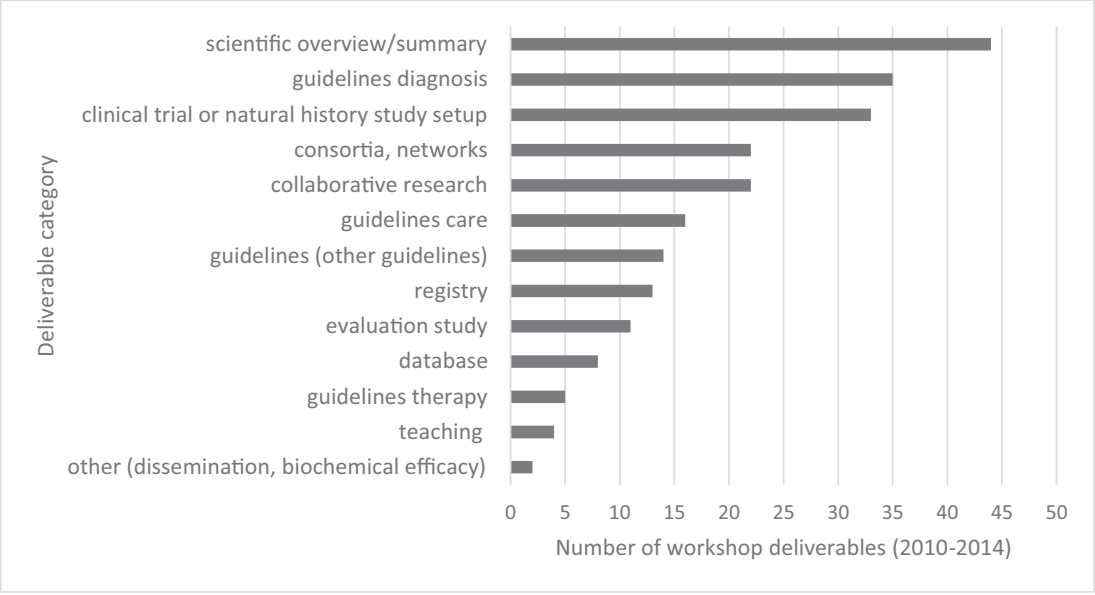

Fig. 7. Number of deliverables per category in the n = 38 workshops evaluated in the survey. The results reflect the workshop deliverables in the period 2010–2014.

generating genetic and pathological insights of a disease, followed by development of treatment and care, including launching of registries to monitor disease or treatment progress. Of the total number of deliverables in this 5-year cohort, 22 (10%) aimed to establish a consortium and another 22 (10%) aimed to set up research collaborations. A good example of a consortium is the Charcot Marie Tooth disease consortium which held nine ENMC workshops in the last 25 years [9].

As stated earlier, we received a response for 29 of the 38 workshops selected. The main outcome of this survey was that almost 90% of all workshop deliverables were achieved: 51% were completed at or directly after the workshop, 33% were started and are still ongoing and 5% were started but delayed (see Fig. 8). Of all deliverables only 8% never started due to several reasons (see below) and for 3% of the 153 deliverables the status of achievement could not be filled in by the respondents.

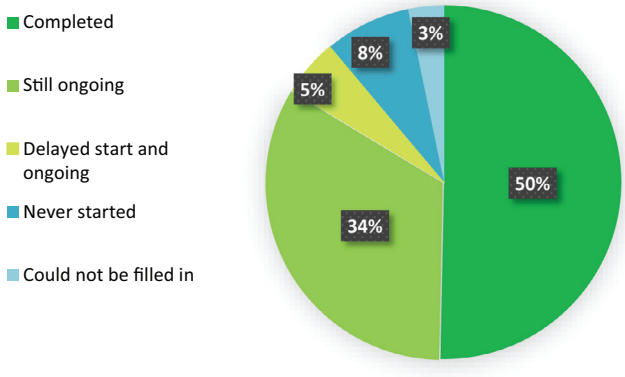

Fig. 8. Rate of ENMC deliverable fulfilment (2010–2014): Almost 90% of the aims defined in the workshop deliverables were achieved.

Of the 77 completed deliverables, 60 were implemented in the broader scientific and professional NMD field (78%), bringing knowledge and new collaborations to the research field, improving designs of clinical (pharma-driven) trials and innovating diagnostic tools for the patients.

Here some examples of comments on implemented deliverables are shown:

"An unexpected deliverable from this workshop was the interaction with the EMA regulators. Before the workshop the field was rather naïve about the regulatory requirements. This 'first contact' resulted in improved interaction with the regulators since this workshop." Prof. Annemieke Aartsma-Rus, about the 194th ENMC workshop [10].

"Our recommended diagnostic tests agreed upon at the workshop are in place in all genetic labs for Myotonic Dystrophy type 2." Prof. Bjarne Udd, about the 180th ENMC workshop [11].

"An overview of natural history studies provided the basis for the follow-up SMA workshop and helped with protocol development for the Roche FIREFISH, AveXis-101 and Ionis/Biogen nursinersen CS3A, ENDEAR, CHERISH and NURTURE studies." Prof. Richard Finkel about the 209th ENMC workshop [12].

4. Discussion

From the Scopus citation index scores, it was clear that ENMC workshop reports are on average well cited in absolute terms. The bulk of reports were cited in a range of 0 to 50 times whereas some individual reports were cited more than 100 times. The major limitation of this citation analysis is that data could not be compared within the NMD literature. Therefore, cluster normalization had to be performed. To obtain a complete view on the impact of ENMC workshops it was, next to the ENMC workshop reports, also interesting to look at indirect publications, which are derived from ENMC workshops. Thus, a large set of papers published within the scientific literature, and spontaneously acknowledging "ENMC", were included in the cluster normalization to find out what the "spin-off" is of workshops and how the network is collaborating. This is the ENMC workshop derived paper set, representing the ENMC ecosystem. ENMC-network papers were well cited when normalized against the neuromuscular field. They showed a 'High Impact' level (MNCS = 1.24) and a 6% higher visibility than average. For scientists and researchers, the high impact level and visibility of this subset of papers shows the added value of ENMC workshop derived papers. The output of ENMC-workshop derived publications was somewhat declining in the beginning of this century but after 2008 the output numbers started to rise again. Impact fluctuates but is high if calculated over the entire period.

A pattern frequently observed by CWTS in collaboration analysis is that the international collaboration publications show a higher impact than other collaboration types. In the ENMC data set, publications by a single institute (and in most papers single author) score even higher impact (MNCS = 1,41). On the other hand, 'International collaboration' scores higher (MNCS = 1,28) than the threshold for 'High Impact' at 1.20, which is still an excellent performance. ENMC strives 'to encourage and facilitate communication and collaboration in the field of neuromuscular research': the high share of 'international collaboration' publications may be interpreted as testimony to a successful implementation of this goal. However the inspiration that participants take home from the workshop and from the contacts with other researchers, and that may results later in a publication assigned to one author only, is also a consequence of the stimulating ENMC workshop environment. Some single author publications were mandatory ENMC workshop reports; in fact, in the past these reports were frequently authored by a single organiser, while today reports are always authored by the team of workshop organisers from various countries and institutes. A high proportion of deliverables of the 5-year cohort was classified to the categories "consortia, network, collaborative research" (see Fig. 7), showing that organisers frequently apply for a workshop with this aim already in mind. Indeed, in most workshop applications at least one of the stated deliverables was dedicated to setting up consortia or other forms of collaborations. Follow-up workshops were also indicative for reaching this crucial aspect of joining forces within neuromuscular research. As an example, the consortium of Charcot-Marie-Tooth disease started at an ENMC workshop in 1997 and had eight follow-up workshops since then [9]. As quoted by Prof. Kannboyina Nagaraju at the ICNMD meeting in 2018: "By bringing experts together ENMC facilitates discussions and by offering the adequate format, ENMC contributes to the progress in research for neuromuscular diseases" [13].

We used the evaluation of 25 years to see how often specific diseases were topic of ENMC workshops. The distribution of disease classes probably reflects the general research effort going on for single diseases („from bench to

bedside“) and additionally it underlines the unmet need of more research initiatives for some much neglected diseases.

The high level of completion and implementation of workshop deliverables shown by the 5-year survey data suggests good workshop performances. One of the future objectives of the ENMC is to repeat this survey analysis in 5 years to monitor the rate of implementation of workshops taking place in the period 2015–2019 and compare the data with the first survey. Workshop organizers learned that a large multidisciplinary group of participants from various specialisms and countries is required to reach consensus. Sometimes it was recognized that a deliverable was too ambitious or outside the reach of the consortium. Sometimes a workshop led to unexpected developments and (patient-driven) turn of priorities. If planned deliverables were not fulfilled, this was due mainly to lack of consensus, resources, time or personnel. ENMC will use this information to help future applicants in securing such measures ahead of the workshop and increase the rate of success."

5. Conclusions

The study showed that ENMC is in line with the first part of its mission: organising workshops that bring experts in the field of NMD together and thereby facilitate the achievement of their goals. ENMC workshops are a starting point for consortia that use the platform successfully to sustain their collaboration over time and write new research proposals, for instance for European grant calls. The topic of workshops is gradually moving from basic science and clinical to more care and cross cutting patient related topics, reflecting the progress in scientific research over the last 25 years. The high citation scores and the impact of workshop derived publications show the degree of collaboration mediated by the workshops and the quality of this collaboration. The high output of publications derived from a workshop (the obligatory report and follow-up publications) represents an added value for researchers and clinicians in being part of the ENMC network.

We are proud that also the second part of our mission – with the aim of improving diagnosis and prognosis, finding effective treatments and optimizing standards of care to improve the quality of life of people affected by neuromuscular disorders – is successfully achieved given the high implementation degree of deliverables. This demonstrates the basic attitude of ENMC workshops, that are not only meant to merely facilitate information exchange but instead to make progress and changes happen.

This research revealed important data for the ENMC strategy: 1. Monitor workshops covering topics or diseases for the coming years, 2. Ensure that future ENMC-derived papers quote properly “ENMC” in order to facilitate future bibliometric analysis and 3. Identify the main challenges in implementing deliverables, in order to better help supporting the success of future workshops and bringing innovations and tools directly to the people affected by a NMD.

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References

[1] Rudel R, Nigro G, Poortman Y. Ten years of ENMC–from a patients' initiative to a successful European research institution: the story of the European Neuromuscular Centre. Neuromuscul Disord 2000;10(1):75–82.

[2] van Engelen B, Zollinger D, Pohlschmidt M, Ambrosini A, Rahbek J. The European NeuroMuscular Centre (ENMC): 20 years on. Neuromuscul Disord 2013;23(4):375–6.

[3] Waltman L, Van Eck NJ. A new methodology for constructing a publication-level classification system of science. J Am Soc Inf Sci Technol 2012;63(12):2378–92.

[4] Subelj L, van Eck NJ, Waltman L. Clustering Scientific publications based on citation relations: a systematic comparison of different methods. PLoS One 2016;11(4):e0154404.

[5] Bushby K, Muntoni F, Urtizberea A, Hughes R, Griggs R. Report on the 124th ENMC International workshop. Treatment of Duchenne muscular dystrophy; defining the gold standards of management in the use of corticosteroids. 2-4 April 2004, Naarden, The Netherlands. Neuromuscul Disord 2004;14(8–9):526–34.

[6] Bushby KM, Beckmann JS. The limb-girdle muscular dystrophies–proposal for a new nomenclature. Neuromuscul Disord 1995;5(4):337–43.

[7] Bushby K, Muntoni F, Bourke JP. 107th ENMC international workshop: the management of cardiac involvement in muscular dystrophy and myotonic dystrophy. 7th-9th June 2002, Naarden, the Netherlands. Neuromuscul Disord 2003;13(2):166–72.

[8] Hoogendijk JE, Amato AA, Lecky BR, Choy EH, Lundberg IE, Rose MR, et al. 119th ENMC international workshop: trial design in adult idiopathic inflammatory myopathies, with the exception of inclusion body myositis, 10-12 October 2003, Naarden, The Netherlands. Neuromuscul Disord 2004;14(5):337–45.

[9] Reilly M. Charcot-Marie-Tooth disease at ENMC workshops. In: 15th International Congress on Neuromuscular Diseases, July 6-10. J Neuromuscul Dis 2018;5(s1):S98–9 Vienna, Austria.

[10] Aartsma-Rus A, Muntoni F. 194th ENMC international workshop. 3rd ENMC workshop on exon skipping: towards clinical application of antisense-mediated exon skipping for Duchenne muscular dystrophy 8-10 December 2012, Naarden, The Netherlands. Neuromuscul Disord 2013;23(11):934–44.

[11] Udd B, Meola G, Krahe R, Wansink DG, Bassez G, Kress W, et al. Myotonic dystrophy type 2 (DM2) and related disorders report of the 180th ENMC workshop including guidelines on diagnostics and management 3-5 December 2010, Naarden, The Netherlands. Neuromuscul Disord 2011;21(6):443–50.

[12] Finkel R, Bertini E, Muntoni F, Mercuri EGroup ESWS. 209th ENMC international workshop: outcome measures and clinical trial readiness in spinal muscular atrophy 7-9 November 2014, Heemskerk, The Netherlands. Neuromuscul Disord 2015;25(7):593–602.

[13] Nagaraju K. ENMC translational research workshop. 15th International Congress on Neuromuscular Diseases, July 6 - 10, 2018 Vienna, Austria. In: J Neuromuscul Dis 2018;5(s1):S99.