



TREAT-NMD Neuromuscular Network

Consultation Report

**Report on the Public Consultation: The Future of
the TREAT-NMD Network**

October 2010



Contents

LIST OF ABBREVIATIONS	3
EXECUTIVE SUMMARY	4
INTRODUCTION.....	5
CONDUCTING THE PUBLIC CONSULTATION	5
KEY FINDINGS.....	7
CURRENT ACTIVITY PRIORITISATION	9
GOVERNANCE	18
FUTURE FUNDING	20
SUMMARY OF RESPONSES.....	23
ANNEXES	24

LIST OF ABBREVIATIONS

CTCC	TREAT-NMD Clinical Trial Coordination Centre
CTSR	Care and Trial Site Registry
EC	European Commission
EMA	European Medicines Agency
FDA	Food and Drug Administration
FP6	Framework Programme 6
NMD	Neuromuscular disease
STAC	Scientific and Technological Advisory Council
TACT	TREAT-NMD Advisory Committee for Therapeutics
TREAT-NMD	Translational Research in Europe for the Assessment and Treatment of Neuromuscular Diseases

EXECUTIVE SUMMARY

The consultation, which ran from 3 September 2010 to 1 October 2010, sought to help define the future activities, governance and funding structures and mechanisms available to the TREAT-NMD Network. Views were sought on which activities should be prioritised and embedded in the future Network and the options for governance structures, stakeholder involvement and possible funding models. The consultation invited views of all stakeholders and interested parties, so the future Network is able to meet their needs.

The response to the consultation was wide ranging, with a global reach, and 430 responses were received via the online questionnaire. It included input from private individuals, academics, clinicians, industry representatives, funding and patient organisations. The majority of respondents agreed that TREAT-NMD had achieved a great deal in the 3.5 years since it began, but reminded that more still needed to be done to ensure further integration of all the relevant groups, and that future funding would be the primary issue facing the Network ahead of the end of the European Commission funding in December 2011.

The activity of the TREAT-NMD Network that received the highest recommendation – with over 90% indicating this as a top priority for the Network, was that of facilitating international collaborations to share data, experience and develop harmonised tools and protocols (Question 5.1). This recognition of what TREAT-NMD can bring to the neuromuscular community recognises the core mission of the Network as well as confirming the key role the Network can bring to the community in the future.

Responses to other current activities as a top priority for the future Network included, biobanking, TACT, clinical trial support through the CTCC, CTSR, patient registries, outcome measures, standards of care and diagnosis, and maintaining a communication infrastructure via the web site and newsletter.

Some activities were also identified as being a secondary priority for the future Network, these included; developing consensus on animal models, neuromyology curriculum, as well as providing a forum for social and ethical issues.

Respondents also indicated support for broadening the focus of the Network to other diseases, groups and organisations, as well as other countries, especially developing countries. This has been an ongoing task of the current Network and will contribute significantly to the future strategy of the Network.

All documents related to the public consultation, including all the responses received, are available on the TREAT-NMD web site at www.treat-nmd.eu/Consultation.

INTRODUCTION

Scope of the consultation

The public consultation sought to solicit comments from the neuromuscular community on how they see the impact of the activities of TREAT-NMD. The consultation also invited comments on the future activities, governance and funding mechanisms available to the network. The consultation is in response to the fact that the current funding for TREAT-NMD from the European Commission will end in December 2011 and the purpose of the consultation was to seek views from stakeholders and interested parties on how TREAT-NMD should define its future activities, strategy and funding sources. Through the consultation process TREAT-NMD aims to:

- Consider whether and how current and newly proposed activities might be embedded and funded in the future network
- Take this opportunity to review the priorities for the future network, and the options for governance structures, stakeholder/partner involvement and potential funding models
- Understand and take into account the views of all stakeholders and interested parties, so that the next stage of TREAT-NMD can meet their needs

Background information regarding the public consultation can be found in Annex A – D.

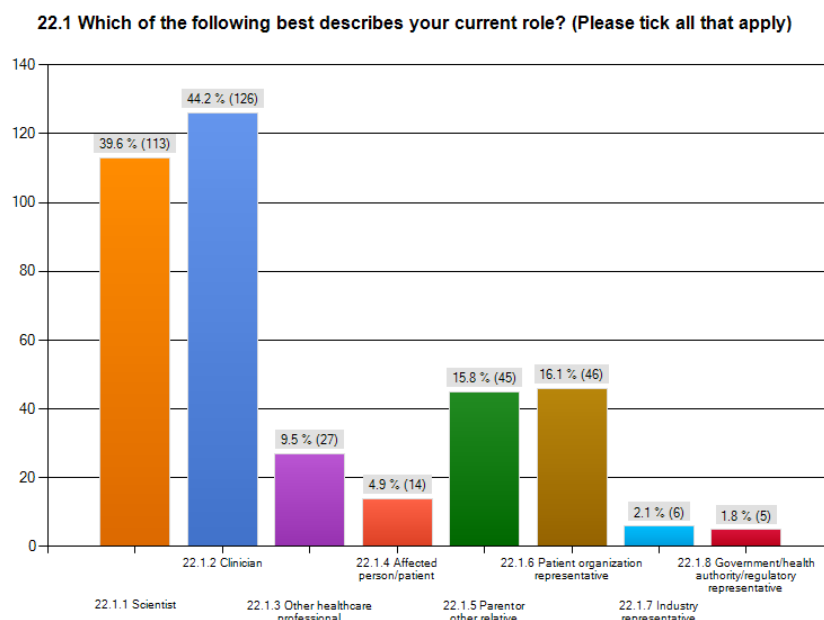
CONDUCTING THE PUBLIC CONSULTATION

Number of responses

The total number of responses to the public consultation was 430. The majority of these responses were from individuals with the remaining representing interested organisations and institutions. The response covers a wide range of participants and a global reach, with over 60 countries worldwide represented and the majority of responses coming from the USA, Germany, Italy and the UK. The consultation did not take into account whether duplication would occur with some submitting more than one response, but as a consultation is not a vote, but a qualitative exercise to collect views and evidence; this is not a cause for concern.

Composition of responses

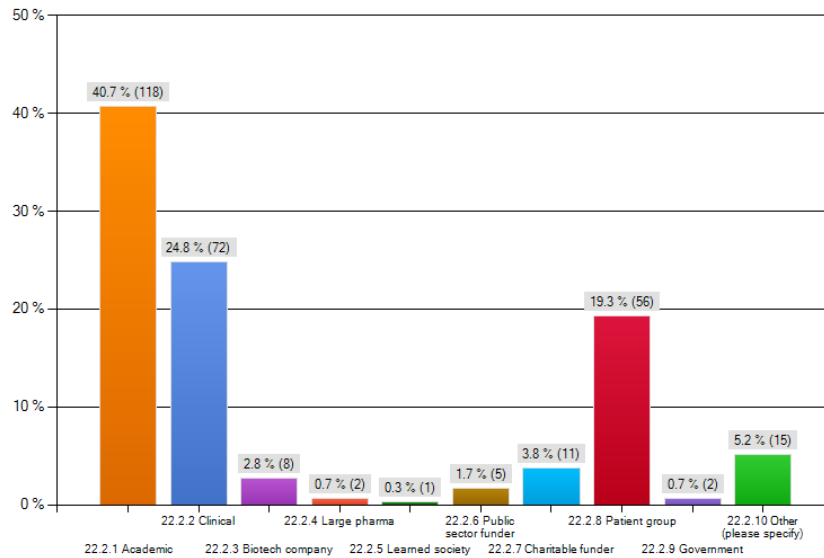
The consultation attracted responses from around the world. Most of the identifiable responses



came from private individuals. Overall, 285 respondents (66.3%) identified their current role with the majority of identifiable responses coming from Clinicians (44.2%) and Scientists (39.6%) (Question 22.1).

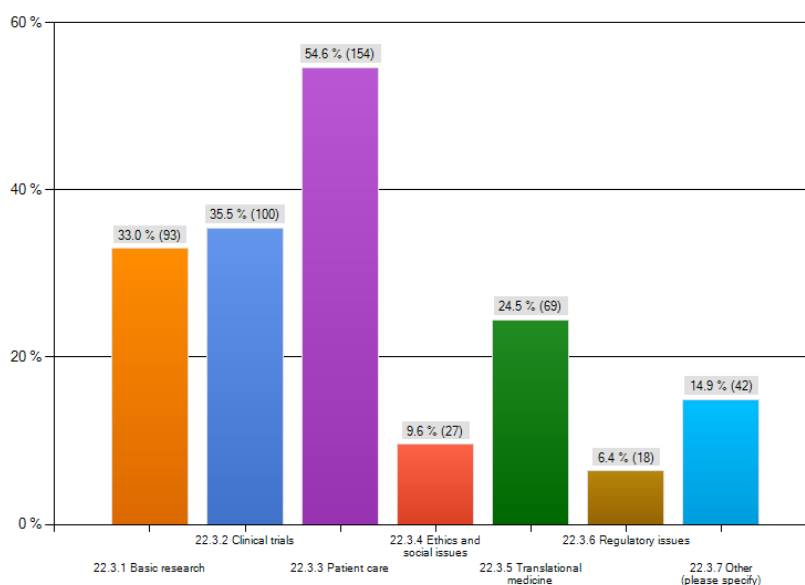
Those respondents were also asked to identify the type of organisation that they represent. Of the 290 respondents (67.4%) the majority were from academic organisations (40.7%) (Question 22.2).

22.2 Which of the following best describes the type of organization you represent? (Please tick one box only)



Respondents were also asked to identify their main area of interest or role with the majority of respondents identifying their role in patient care (from a total of 282 respondents who answered this question 54.6% identified patient care) (Question 22.3).

22.3 Which of the following best describes your current role? (Please tick all that apply)

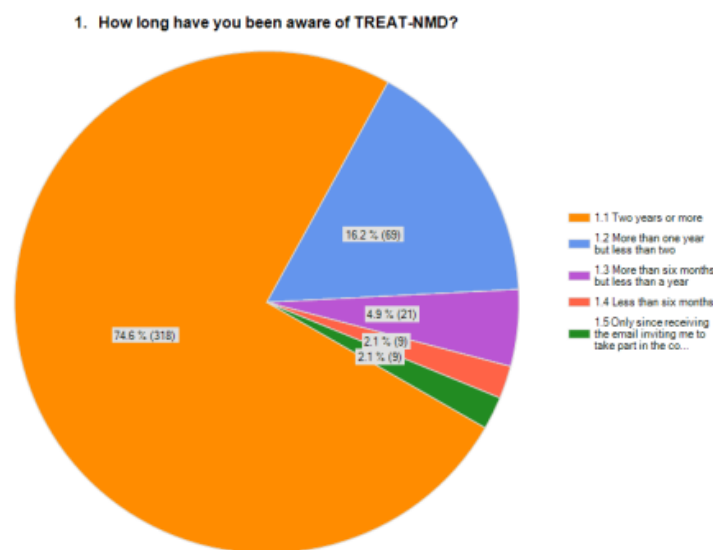


While the numbers and broad breakdown of the types of respondent are helpful for demonstrating the degree of interest in and commitment to the Network and its activities, as well as any concerns they may have, there are also limitations on their usefulness, as not all respondents completed the questions regarding their role and organisation. Therefore, to determine whether any one group of respondents identified in the consultation would have introduced bias we analysed the responses to a number of activities to determine the proportion of each group who completed the activity prioritisation. Across those activities analysed the responses of each group was representative of the overall proportion of respondents illustrated by Question 22.1 above.

The submitted responses are helpful in analysing what the community’s main underlying concerns are, and what sort of activities would be best addressed in the future Network. The key points highlighted by the respondents are described in the next section (Key Findings) as well as in the Activity Prioritisation section.

KEY FINDINGS

The consultation invited respondents to give their views on the TREAT-NMD Network to date, current activities and future focus. Awareness of TREAT-NMD among the respondents was high with 387 (90%) respondents having been aware of TREAT-NMD for more than one year (426 total responses from a possible 430).



Respondents were then asked to list the main significant achievements of TREAT-NMD to date, which included:

- Providing a network to bring people together, so promoting international collaboration and a coordination of effort, while avoiding duplication of effort
- Setting up, promoting and advising on patient registries, standards of care, best practice and outcome measures
- Providing a source of news, information and resources whilst raising the general profile of neuromuscular diseases

- Supporting and facilitating new studies and trials

The consultation also asked respondents what TREAT-NMD could have done better. Responses included:

- Better communication with and involvement of patients with more information in lay terms and in other languages
- Making TREAT-NMD more visible and more transparent
- Broaden the network to include other diseases, other groups and organisations, and other countries, especially developing countries
- Provide support and training for young researchers/clinicians and funds (or advice on finding funds) for research

Overall, respondents were very satisfied that TREAT-NMD had done a 'good job' and that it should continue.

Respondents were also asked to identify what future obstacles could face the Network. Responses included:

- Lack of funding (the most common obstacle stated by respondents) balanced against the need to remain independent (especially from drug companies)
- The network becoming fragmented due to lack of cooperation between different groups and countries because people are in competition with each other and not prepared to share resources and ideas
- Lack of standard practices in ethics, research and treatment between different countries

CURRENT ACTIVITY PRIORITISATION

The consultation invited respondents to prioritise the current TREAT-NMD activities in order to help define the future activities and focus of the Network.

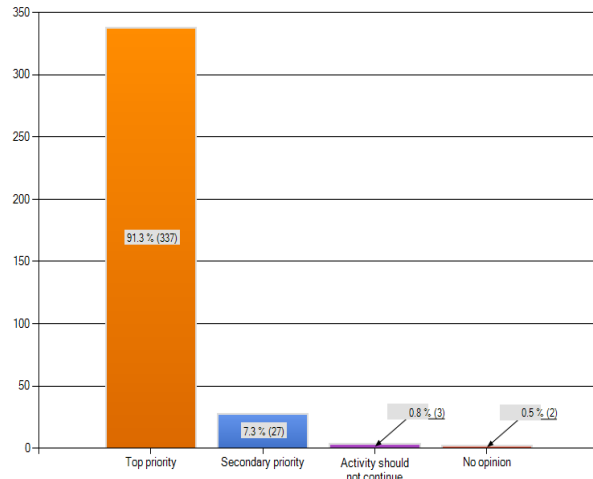
Facilitating International Collaborations

Facilitating international collaborations between research groups to share data and experience and develop harmonized tools and protocols. In total, 369 (85.8%) respondents answered this question.

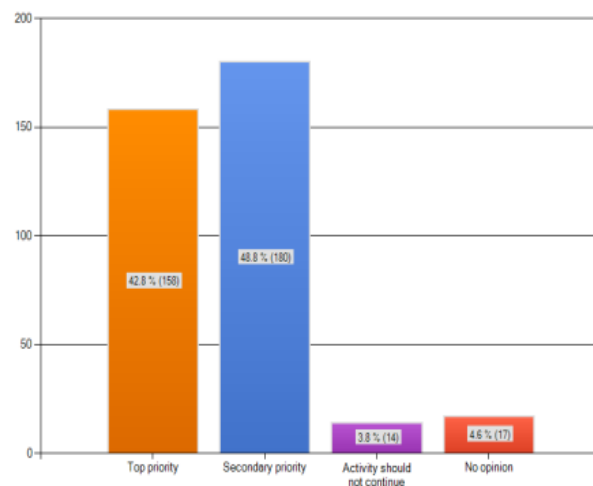
Evaluating existing animal models for NMDs, developing international consensus on their use and creating standard operating procedures (SOPs) to allow standardized assessment across labs. 369 (85.8%) respondents answered this question with 23 commenting that the work had been achieved, duplicated, or should not be the sole responsibility of TREAT-NMD.

Facilitating global exchange of biomaterials for research via an online database of DNA, cell and tissue samples (EuroBioBank). 367 (85.3%) respondents answered this question with 15 commenting that the work had been achieved, duplicated, or is too expensive for TREAT-NMD to maintain.

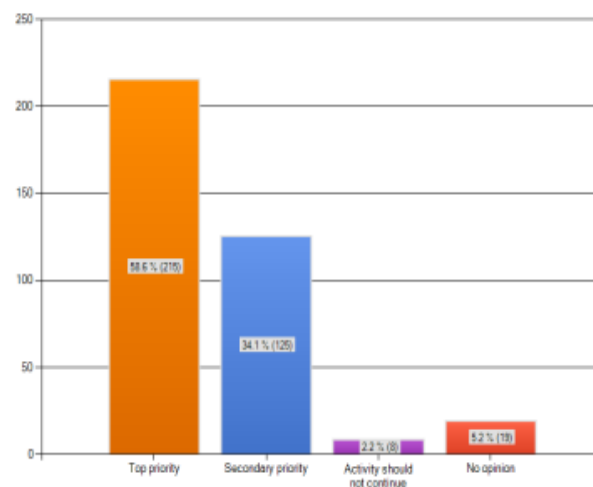
Question 5.1 - Collaborate between research groups



Question 5.2 - Animal Models



Question 5.3 - EuroBioBanks

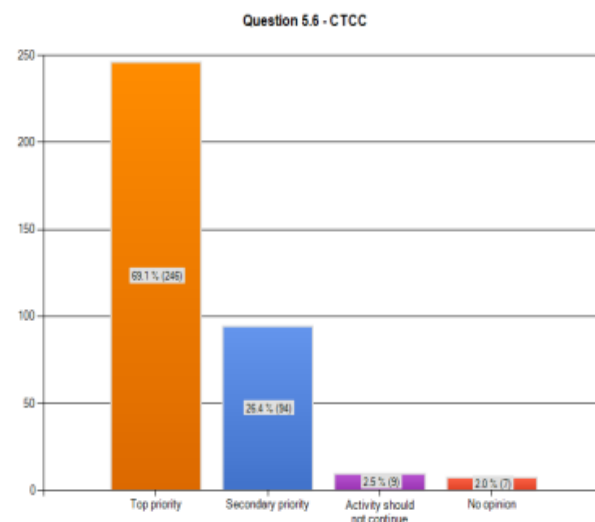
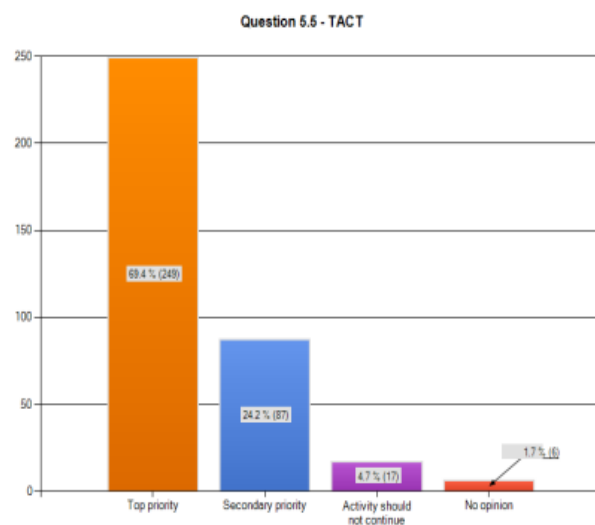
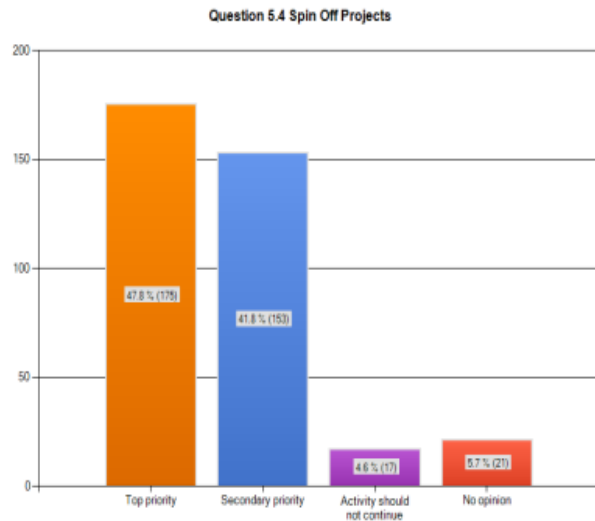


Acting as a platform for spin-off projects (bringing participants together in the set-up phase, assisting in proposal-writing and acting as a communication and dissemination platform). 366 (85.1%) respondents answered this question with 17 commenting that the work duplicates other efforts and does not require oversight from TREAT-NMD.

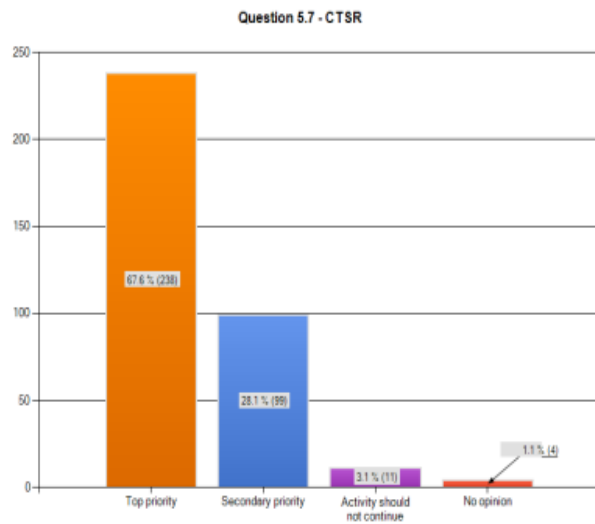
Creating Clinical Trial-Readiness

Providing expert evaluation and advice to researchers on the steps to be taken to take promising drugs and therapeutic targets through to clinical trial through the TREAT-NMD Advisory Committee for Therapeutics (TACT). 359 (83.5%) respondents answered this question with 19 commenting that this duplicates other initiatives.

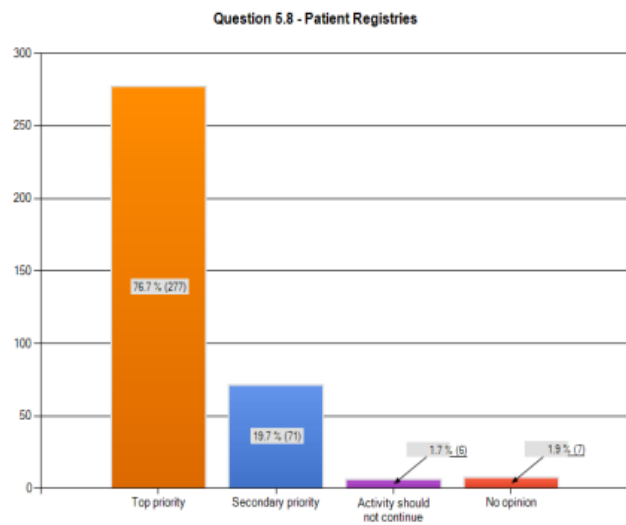
Providing expert support services for neuromuscular clinical trials, advising researchers, companies or CROs through the Clinical Trial Coordination Centre (CTCC). 356 (82.8%) respondents answered this question with 15 commenting that this duplicates other initiatives or that industry would not use this service.



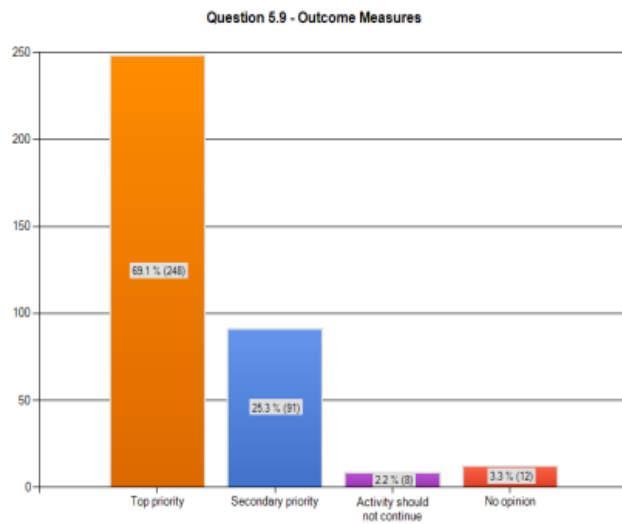
Developing a global registry of care and trial sites specializing in NMDs including information about their facilities through the Care and Trial Site Registry (CTSR). 352 (81.9%) respondents answered this question with 15 commenting that this duplicates other work and is difficult to provide real quality assurance.



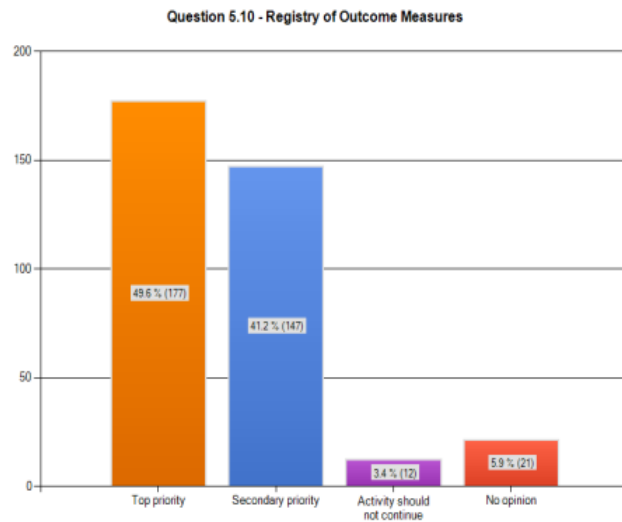
Developing patient registries to facilitate patient recruitment for trials and provide feedback to patients on developments relevant to their condition. 361 (84%) respondents answered this question with 12 commenting that this work has been achieved or duplicated but should still provide more feedback and coordination with other efforts – it should remain one of our top priorities.



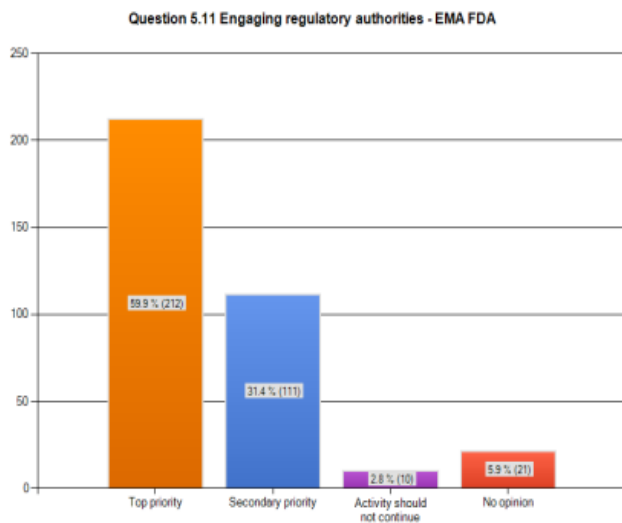
Developing and validating outcome measures appropriate for clinical trials in different conditions. 359 (83.5%) respondents answered this question with 13 commenting that this work has already been achieved or duplicated and coordination with other groups is important.



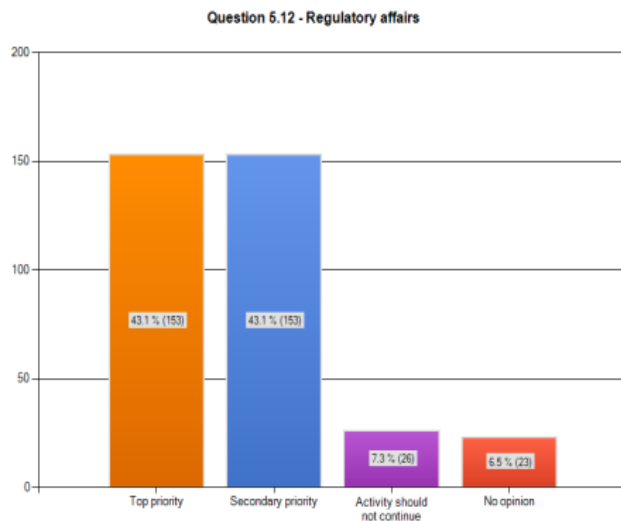
Developing an online Registry of Outcome Measures (ROM) with active user input to facilitate access to existing outcome measures. 357 (83%) respondents answered this question with 18 commenting that this work has already been achieved and should be maintained and updated only.



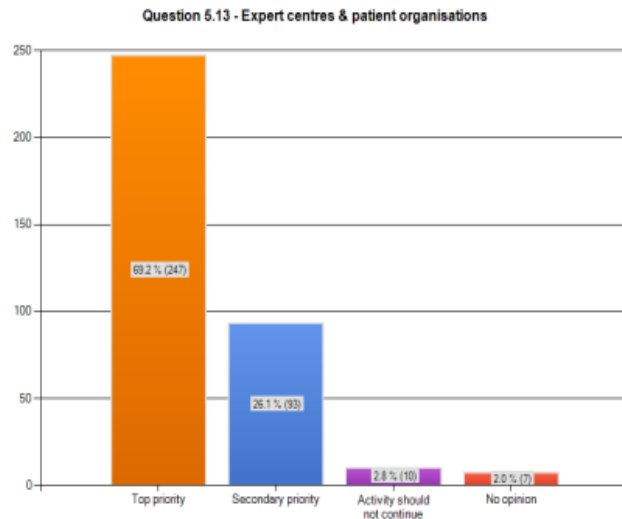
Engaging the regulatory authorities, such as EMA and FDA, in strategic dialogue on areas of key concern to NMDs. 354 (82.3%) respondents answered this question with 14 commenting that this work is duplicated and that TREAT-NMD should collaborate more with patient organisations on this activity.



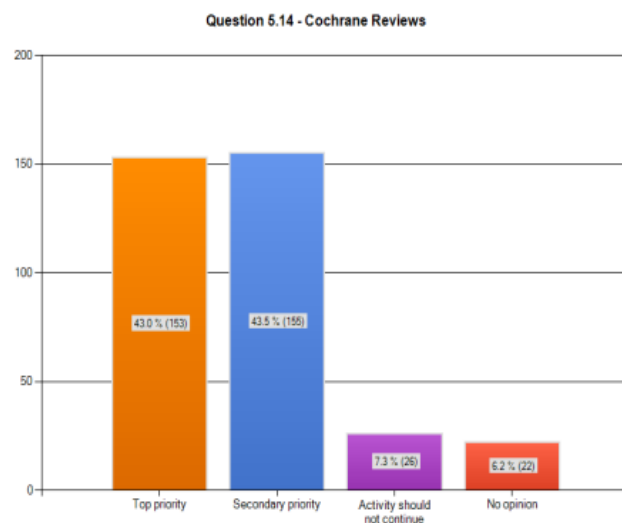
Maintaining a database of regulatory requirements for clinical trials in different countries (Regulatory Affairs Database). 355 (82.6%) respondents answered this question with 27 commenting that this duplicates other efforts and this should be the responsibility of the regulatory authorities and industry.



Supporting national networking of expert centres and patient organizations in countries worldwide with the aim of improving care standards and facilitating trial-readiness. 357 (83%) respondents answered this question with 12 commenting that this duplicates other work that naturally occurs across the field.

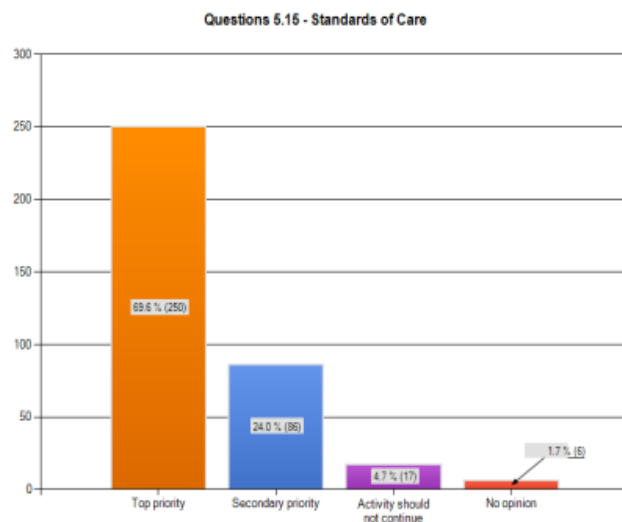


Undertaking and updating systematic reviews of published and unpublished clinical trials (Cochrane reviews). 356 (82.8%) respondents answered this question with 29 commenting that this duplicates other work and should be completed by the Cochrane Group.

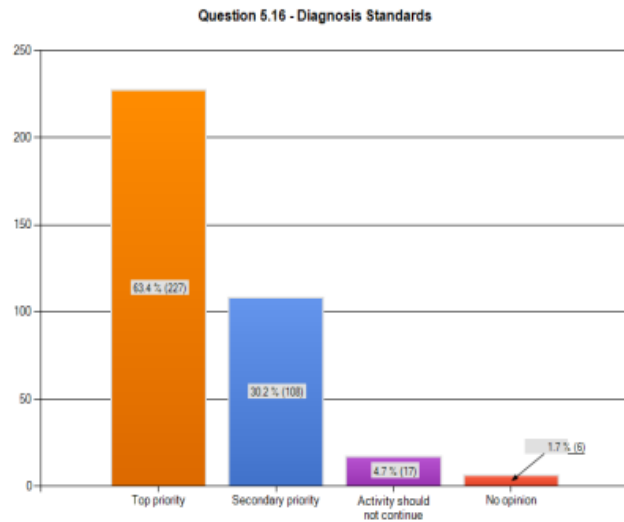


Improving Care and Diagnosis

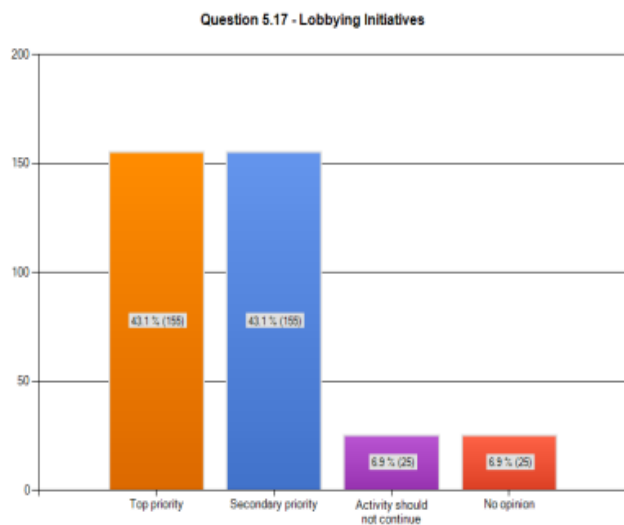
Developing and implementing international consensus guidelines on standards of care for NMDs. 359 (83.5%) respondents answered this question with 24 commenting that development and implementation should be done in partnership with patient organisations.



Developing and implementing international consensus guidelines on diagnostic standards and techniques for NMDs. 358 (83.3%) respondents answered this question with 23 commenting that this duplicates work done elsewhere.

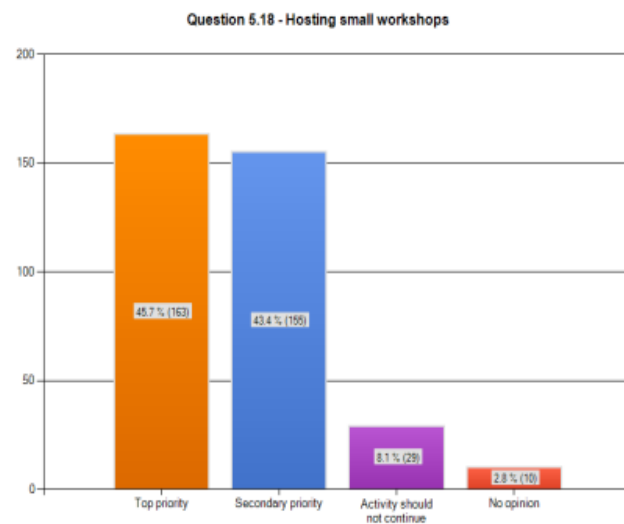


Support lobbying initiatives (by providing expert opinions and information on best practice to influence national healthcare and research decisions). 360 (83.7%) respondents answered this question with 27 commenting that this duplicates work elsewhere and should not be the role of the Network, but is a core activity of patient organisations.

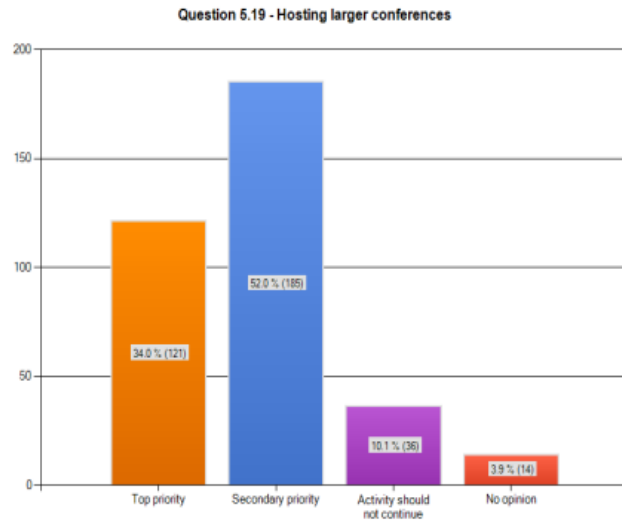


Trainings, Conferences and Workshops

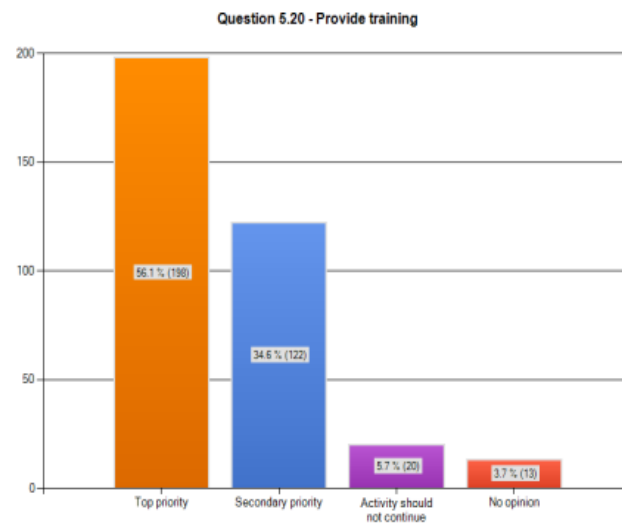
Hosting small focused workshops on a particular area where progress is required. 357 (83%) respondents answered this question with 34 commenting that this duplicates work elsewhere, such as the ENMC workshops.



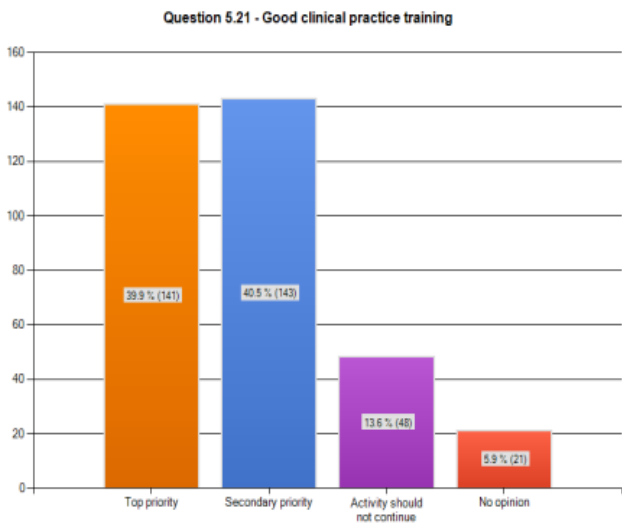
Hosting larger international conferences on broader issues in translational research in NMDs. 356 (82.8%) respondents answered this question with 46 commenting that this duplicates work elsewhere.



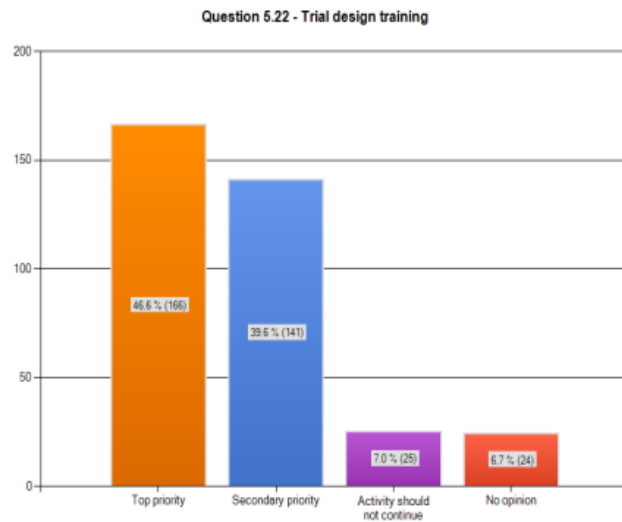
Providing expert training on NMDs to healthcare professionals in countries with poorer access to specialist services. 353 (82.1%) respondents answered this question with 28 commenting that this duplicates some national initiatives and could also be too expensive to sustain through the Network.



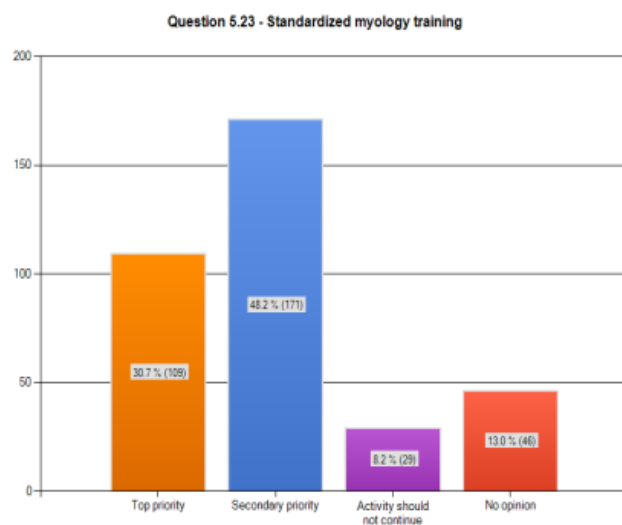
Providing good clinical practice (GCP) training. 353 (82.1%) respondents answered this question with 54 commenting that this duplicates work elsewhere and should be provided locally.



Providing specialist training on trial design (special focus on NMD trials and the challenges of trials in small populations). 356 (82.8%) respondents answered this question with 28 commenting that this duplicates work elsewhere and may be too expensive for the Network.

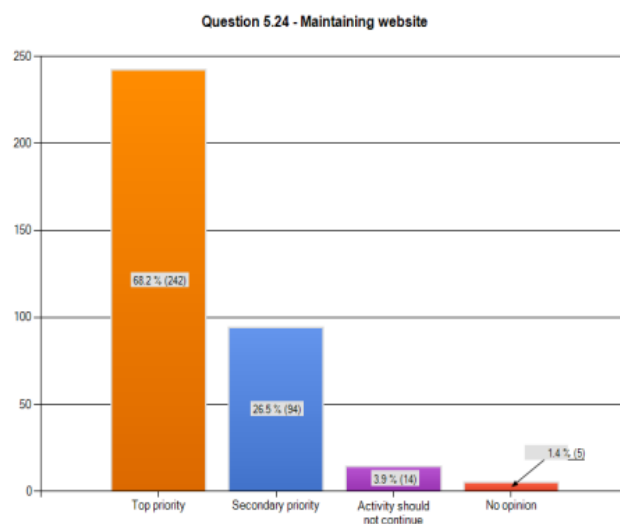


Developing a standardized curriculum for specialist medical training in myology in Europe. 355 (82.6%) respondents answered this question with 36 commenting that this duplicates work elsewhere and should not be part of the focus of the Network.

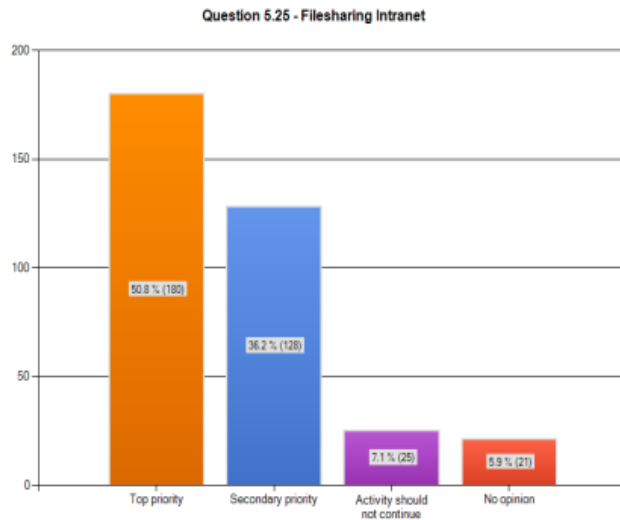


Communication

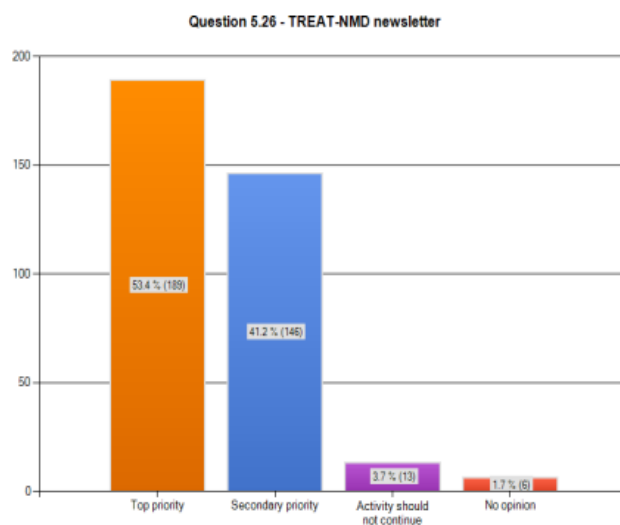
Maintaining a website providing independent expert information for patients and families, healthcare professionals, researchers, and industry. 355 (82.6%) respondents answered this question with 20 commenting that this duplicates work elsewhere or has already been achieved.



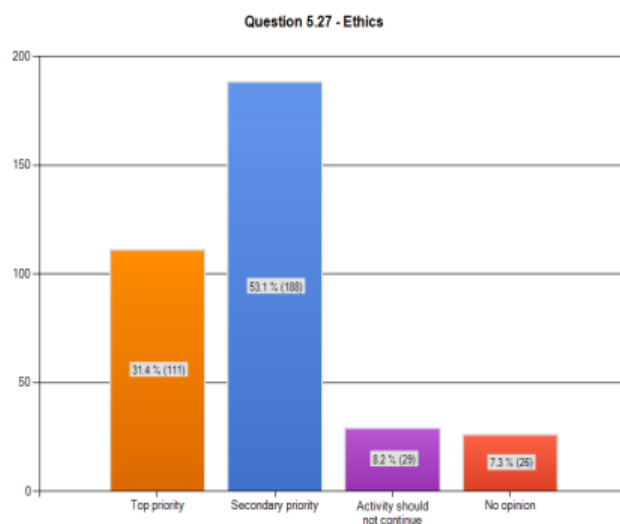
Providing online tools for collaboration between research groups (intranet, file sharing etc.). 354 (82.3%) respondents answered this question with 29 commenting that this type of resource is not necessary.



Providing updates by means of regular newsletters. 354 (82.3%) respondents answered this question with 13 commenting that the frequency of newsletters should be decreased.



Providing a forum for discussion and debate on social and ethical issues for both patients and professionals. 354 (82.3%) respondents answered this question with 30 commenting that this duplicates work elsewhere and is not particularly useful or necessary.



Respondents also identified other activities that TREAT-NMD should consider as part of the future focus of the Network. These include:

- Lobbying
- Training, especially for developing countries and including broadening registries
- Help with funding both for research and for patient groups
- Focus on quality of life of patients including advice on equipment, a summary and evaluation of therapies and supplements and advice on impacts of exercise

TREAT-NMD Tools and Services – providing a service to the community

The consultation also asked respondents if the current TREAT-NMD tools, services and infrastructure are useful in supporting the needs of the neuromuscular community, such as the development of roadmaps across the spectrum of diseases to help address current unmet needs. 264 (61.4%) respondents answered this question with 90.2% of respondents agreeing that the current tools, services and infrastructure are useful, but they need to be further enhanced and better integrated to support a wide variety of neuromuscular diseases.

One of the aims of TREAT-NMD is to promote international research collaborations and make it easier for different groups to share information and work together. Respondents were asked what additional specific measures TREAT-NMD should put in place to facilitate effective international research collaborations in the future. Responses included:

- Grants and assistance in setting up exchange of personnel between different countries to specifically support and promote internationally collaborative projects
- More organisation of conferences/meetings/workshops/training internationally
- Training and advice for developing countries and smaller groups

GOVERNANCE

TREAT-NMD is currently funded by the European Commission (EC) until December 2011. Under the current structure TREAT-NMD is managed by a central Coordination Office based at Newcastle University, under the coordination of Professor Kate Bushby and Professor Volker Straub. The Project Coordinators are also ultimately responsible for ensuring the TREAT-NMD Network achieves all its deliverables as laid out in the current funding contract with the EC. In addition to this coordination role TREAT-NMD is also currently managed by a Governing Board made up of representatives from each funded partner in TREAT-NMD and are responsible for both the delivery of the aims and objectives of the current Network, as well as overseeing its strategic direction and governance.

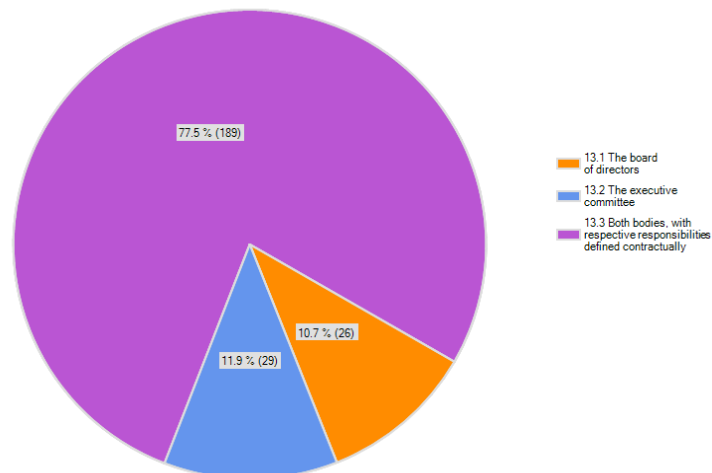
The current Governing Board is supported by advisory councils and committees made up of partners and external experts (these include Scientific and Technological, Industrial Liaison, Intellectual Property and Use, and Project Ethics). The Scientific and Technological Advisory Council (STAC) meets in person on an annual basis to review the progress of the TREAT-NMD Network and to offer recommendations to the TREAT-NMD Governing Board on activities related to the objectives of the Network.

In the consultation it was proposed that the governance of the future network could consist of either a Board of Directors and/or an Executive Committee that would oversee responsibility for all activities and resources. The two-tier option of having an Executive Committee that reports to a Board of Directors would need to define their separate roles and responsibilities contractually in

order to define a clear governance structure with clear policies and procedures and lines of communication.

The consultation asked **“One possible model for the future (governance) structure envisages the creation of a board of directors to provide oversight and an executive committee made up of representatives of the institutions carrying out the work. In such a model, should strategic decisions be taken by the Board of Directors, the Executive Committee, or both bodies, with respective responsibilities defined contractually?”** In total 244 (56.7%) respondents answered this question with 77.5% of these respondents opting for both bodies to be involved in the future governance structure (Question 13).

13. One possible model for the future structure envisages the creation of a board of directors to provide oversight and an executive committee made up of representatives of the institutions carrying out the work. In such a model, should strategic decisions be taken by:



Comments on this dual model of governance suggested that this would allow for more appropriate oversight, with checks and balances, of activities with the Executive Committee responsible for day-to-day monitoring and the Board of Directors responsible for agreeing on the overall strategy and direction of the network and its activities.

Respondents were asked what specific measures TREAT-NMD should put in place to ensure its stakeholders have an adequate voice at a strategic level. Responses included:

- Encourage and act upon feedback or voting, publish results of feedback or votes and do it before decisions are made
- Define stakeholders and roles clearly and have rotating roles possibly involving more local ‘branches’ or offices
- Better and wider representation of patient groups
- Stakeholder meetings/workshops

Respondents were asked what specific measures TREAT-NMD should put in place to ensure it retains its current independence. Responses included:

- Accept no sponsorship from pharmaceutical companies but instead seek funding from public sources, government, the EU or through donations.
- Funding should come from a range of different sources with a variety of people on the committee so that no one group has overall control or undue influence
- Put in place a clear and transparent reporting process to include external auditing

Respondents were asked what specific measures should TREAT-NMD put in place to ensure active ideas generation can come from anywhere within the neuromuscular field. Responses included:

- Provide and promote an award, prize or grant for the best new ideas
- Have an ideas box on the internet as well as an forum online which is well publicised and clearly explained
- Use meetings, conferences and symposia to have brainstorming sessions and collect ideas

Respondents were asked what specific measures should TREAT-NMD put in place to ensure its initiatives are well integrated and harmonized with those of other stakeholders active in the field. Responses included:

- Have open communication channels and listen to the opinions aired
- Encourage and support partnerships and cooperation between groups which includes sharing of data and information
- Involve all the key/best labs, people and patient groups and those from developing countries and smaller organisations

FUTURE FUNDING

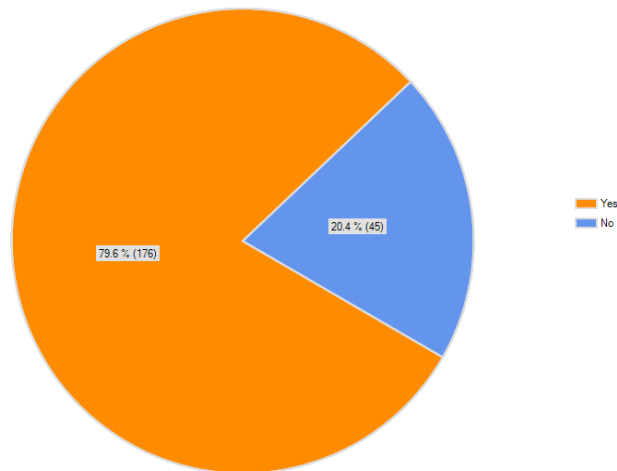
Funders such as advocacy groups and charitable foundations working in the neuromuscular area have indicated a willingness to contribute funds towards the continuation of TREAT-NMD. Respondents were asked what rights/benefits such funders should expect as a return on their investment. Responses included:

- Clear, regular feedback on the achievement of objectives, progress and how the money has been spent
- Recognition or publicity but nothing else (some said none at all)
- Many agreed that funders should be able to specify in what area their money was to be spent
- Inclusion of their opinions and an input into the strategic direction of TREAT-NMD and representation on the board (although many said it should be without voting rights)

Respondents were asked whether there is a need to enable organizations to provide project-specific funding targeted at individual TREAT-NMD activities. Of the 221 respondents who answered this question 79.6% agreed that we should allow targeted funds (Question 16.2). The main responses on how this should be achieved included:

- Those who said 'Yes' think that funders should be able to target their money to a specific project but not at the expense of other areas of TREAT-NMD. This would encourage more supporters
- This should be a decision for the Board to decide on a case by case basis
- Those who responded 'No' (this was a small but significant minority) claimed it would penalise more novel research and smaller areas of interest

16.2 Is there a need to enable organizations to provide project-specific funding targeted at individual TREAT-NMD activities?



Respondents were asked how might funding to sustain the overall infrastructure and services, including management, coordination and administrative activities, be obtained. Responses included:

- National governments and the EU, charities and industry
- Implementing membership fees perhaps in proportion to the size of an organisation
- Through submission of grant applications
- Providing chargeable services

TREAT-NMD already receives a small amount of industry funding, primarily as a service cost for access to registry data. This money has gone towards the costs of sustaining activities not covered by the EU funding (for example covering costs for participation in training meetings). Respondents were asked if industry should be charged for these and other services. Of the 218 respondents who answered this question the responses were:

- Yes (<80% of respondents) but with some rules (not too much, and only if no conflict of interest)
- Unsure >5%
- No >15%

Respondents were asked if industry should sponsor meetings. Of the 217 respondents who answered this question the responses were:

- Yes (80%), again with some conditions (no strings, and no undue influence)
- Unsure/No 20%

Respondents were asked whether it would be appropriate for industry to contribute to the future funding of TREAT-NMD in other ways. If so, which? If not, why not? Of the 163 respondents who answered this question the responses were:

- Yes (80%) – For services, clinical trials, basic research and for training. However, they should not be the main source of funding and they should have no influence on the governance of the network

- No/unsure (20%) – There is a potential conflict of interest and TREAT-NMD may lose its current independence

Respondents were asked how TREAT-NMD should further develop its services to industry. Responses included:

- Ask industry what they need and be responsive to that – there is no point in duplication or offering things they don't want/need
- Provide consultancy, advice (including TACT) and training for personnel
- Enable access to registries

Membership of TREAT-NMD

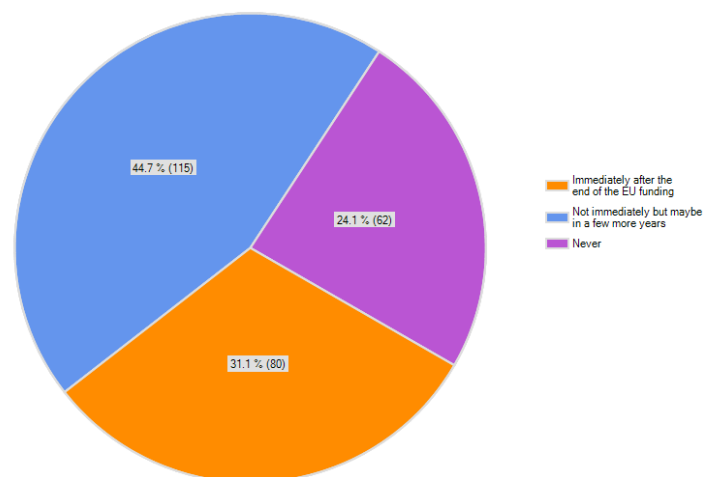
The TREAT-NMD Network currently has 22 partners across 11 European countries that are funded by the EC, but it was recognised that there are many other organisations, institutions, companies and individuals who are doing much valuable work in the neuromuscular field and who might benefit from closer links with TREAT-NMD. While 'partnership' in the network is restricted to those who have signed the contract with the EC to perform specific tasks within the network, 'membership' is currently open to all interested parties and is free of charge.

All current applicants for membership must be willing to adhere to our 'Members' Charter', which is available on the TREAT-NMD web site. During this current phase Members benefit from closer ties with the network and we are working together with our members to help implement and disseminate the network's goals, such as improved patient care through to specialist scientific training.

In order to support operating costs the consultation asked **“Should TREAT-NMD consider initiating membership fees to cover some of its operational costs (as many professional societies and charitable bodies do)?”**

Of the 257 (59.8%) respondents who answered this question 75.9% agreed that membership fees should be introduced immediately after or within a few years of the end of the EU funding period (Question 18.1). Comments received from respondents thought that any fees introduced should not restrict participation in the Network and that a tiered approach to fees could ensure that individuals and larger organisations and institutions would pay an appropriate membership fee to help subsidise operating costs. This fee structure would need to be investigated further to ensure that access for all is maintained. The introduction of membership fees would also have to show a clear return and benefit to members, besides supporting the ongoing operational needs of the Network, and this would need to be discussed before the implementation of any future membership policy.

18.1 Should TREAT-NMD consider initiating membership fees to cover some of its operational costs (as many professional societies and charitable bodies do)?



SUMMARY OF RESPONSES

The following section highlights some of the key issues which were raised by a number of respondents.

To date TREAT-NMD has accomplished a great deal, especially in regard to patient registries, clinical guidelines and providing timely and useful information. It was also recognised that in the short time the Network has been active significant progress had been made, but respondents were keen to see further involvement of patient organisations, from planning through to execution of activities, and that other diseases should be integrated further into the Network. However, the main obstacle to continuation of the Network is funding and whether this should be sourced from government or through stakeholders, or a combination of both. The issue of funding would dictate the willingness of stakeholders to continue to work in the Network.

To ensure the future sustainability of the Network it is important that a clear, focussed strategy is defined with clear goals and objectives, and an oversight or governance structure that allows stakeholders to steer this strategy and implement the activities effectively and efficiently.

ANNEXES

A. Background to the Consultation

TREAT-NMD is currently an EC-funded 'Network of Excellence' with the aim of addressing the fragmentation currently hindering translational research for cutting edge therapies in rare neuromuscular diseases. TREAT-NMD was funded with €10 million from the EC (through Framework Programme 6; FP6) from January 2007 to December 2011. The rules of a Network of Excellence preclude renewal funding from the EC but endorse the establishment of a durable organisation after December 2011.

TREAT-NMD has developed a truly international network that has provided a number of tools and resources that aim to accelerate therapy development and delivery for neuromuscular diseases. TREAT-NMD also serves as a platform to develop these resources and tools together with other groups and organisations to enable continued support for therapy development.

TREAT-NMD is currently managed by a central Coordination Office based at Newcastle University and under the supervision of Project Coordinators Professor Kate Bushby and Professor Volker Straub. The Project Coordinators are responsible for ensuring the TREAT-NMD Network achieves all its deliverables and milestones as laid out in the contract with the EC. In addition to the Project Coordinators TREAT-NMD is also managed by a Governing Board made up of representatives from each funded partner in the TREAT-NMD consortium, who not only deliver the aims and objectives of the current Network but also oversee its strategic direction and governance.

The Governing Board is supported by advisory councils and committees made up of partners and external experts (these include Scientific and Technological, Industrial Liaison, Intellectual Property and Use, and Project Ethics). The Scientific and Technological Advisory Council (STAC) meets in person on an annual basis to review the progress of the TREAT-NMD Network and to offer recommendations to the TREAT-NMD Governing Board on activities related to the objectives of the Network.

The progress of the TREAT-NMD Network is reviewed on an annual basis by the EC and through an external reviewer appointed by the EC, who reports on whether the project has fully achieved its objectives and technical goals for the period and makes recommendations to the EC.

The consultation document points out that TREAT-NMD wants to serve as a platform for all stakeholders in the neuromuscular field to deliver on our joint objectives. TREAT-NMD is NOT a patient organisation and will NOT seek funds from the public directly. TREAT-NMD does NOT seek to fund basic research or clinical trials, and is NOT tied to one particular industrial partner. However, TREAT-NMD can support the aims of other organisations to implement translational research and delivery of therapies and care for patients worldwide.

Questions on which views were sought

The consultation invited respondents to give their views on a number of questions, set out below:

What do you see as TREAT-NMD's most significant achievements so far?

What could TREAT-NMD have done better?

What do you see as the main obstacles to TREAT-NMD's continuation in the future?

Please indicate the future level of priority of each of the currently funded activities.

What other activities not mentioned above should TREAT-NMD focus on in future? Please detail the activity and explain why TREAT-NMD is the appropriate body.

What specific measures should TREAT-NMD put in place to ensure its stakeholders have an adequate voice at a strategic level?

What specific measures should TREAT-NMD put in place to ensure it retains its current independence?

What specific measures should TREAT-NMD put in place to ensure active ideas generation can come from anywhere within the neuromuscular field?

What specific measures should TREAT-NMD put in place to ensure its initiatives are well integrated and harmonized with those of other stakeholders active in the field?

What governance structure should TREAT-NMD adopt?

What rights/benefits should such funders expect as a return on their investment?

Is there a need to enable organizations to provide project-specific funding targeted at individual TREAT-NMD activities?

How might funding to sustain the overall infrastructure and services, including management, coordination and administrative activities, be obtained?

Should TREAT-NMD ask industry to pay for services?

Should industry sponsor meetings associated with TREAT-NMD?

Would it be appropriate for industry to contribute to the future funding of TREAT-NMD in other ways? If so, which? If not, why not?

How else could TREAT-NMD develop its services for industry?

Should TREAT-NMD consider initiating membership fees to cover some of its operational costs (as many professional societies and charitable bodies do)?

The current activities of TREAT-NMD are defined in the contract with the EC, however, it is recognised that the future Network should focus its efforts on those areas which best support and serve the needs of the neuromuscular community. This focus should complement and not duplicate current initiatives and should provide added value to the efforts of the community. Through establishing ongoing collaborations and partnerships the TREAT-NMD Network aims to provide a cost-effective platform that is committed to accelerating therapy development and delivery for the benefit of all patients worldwide. By expanding the current level of partnerships it is envisioned the Network can bring a focus to the issues facing effective and efficient delivery of new therapies.

How the consultation was carried out

The consultation questions were defined with input from the Network partners and a number of stakeholders, which are available as Annex B of this report. This document also includes the summary data from the consultation.

The consultation period began on 3 September 2010, with the publication of a Consultation Document, and ran until 1 October 2010. Every effort was made to bring the consultation to the attention of all those for whom it was intended, by dissemination of the consultation document and appropriate web links through the newsletter subscriber list, the TREAT-NMD web site, and through direct communication with individuals and groups with a known interest in the Network. Recipients were encouraged to disseminate and increase awareness of the consultation through their own contacts and networks.

The Consultation Document described the scope of the consultation and the current activities and structure of the Network, as well as relevant background information in order to make the document self-contained. The Consultation Document is available as Annex C of this report.

The consultation document and newsletter announcements explained how to become involved in the consultation. Web links and downloadable documents were provided for responses by email or online. Participants were encouraged to describe their views as fully and openly as possible.

All participants were informed that their responses would be made publically available on the TREAT-NMD web site; however, we would not publish any identifiable information or accredit any responses with any personal information. All published responses or comments would be accredited by the respondent's role only. Of the 430 responses received, 236 provided either their name and/or email address (the remaining 194 skipped these questions). The responses received during the consultation period are available to view on the TREAT-NMD web site (Annex D).

B. Consultation Questionnaire (with summary data) can be found via the link:

www.treat-nmd.eu/ConsultationResponsesSummary

C. Consultation Document can be found via the link:

www.treat-nmd.eu/TREAT-NMD_Consultation_Document_Sept2010.pdf

D. Spreadsheet of all responses received can be found via this link:

www.treat-nmd.eu/Consultation_Responses_Complete