



TREAT-NMD Neuromuscular Network

Summer / Spring 2010 Newsletter for Europe

Welcome to this newsletter of the TREAT-NMD network especially for patient organisations. This edition is the first one that will be send out to all patient organisations and patient groups in Europe and beyond.

TREAT-NMD is a network for people with neuromuscular diseases and professionals working in the field. It aims to advance diagnosis and care and develop new treatments for the benefit of patients and families, working closely with scientists, healthcare professionals, the pharmaceutical industry and patient groups around the world. TREAT-NMD does have projects that are important for patients, patients organisations, physicians, researchers and industry to know about. These projects are important tools to accelerate treatment for neuromuscular diseases. We need your support to make these projects to work optimal and to reach our joint goal. You can actively participate and tell your physicians and researchers about us and our projects.

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Become a Member of TREAT-NMD

If you are interested in becoming a member of the TREAT-NMD Network please visit our web site to download our membership charter. Membership is free of charge. An application form is also available for download. The web link to our Members' section is: <http://www.treat-nmd.eu/research/get-involved/>.

We look forward to welcoming new members!

About this newsletter

This newsletter is sent to all patient organisations and patient groups in Europe. Earlier editions of the regular newsletter can be found online at our web site. If you would like to subscribe directly, please visit our website at www.treat-nmd.eu/ where you will find a subscription form at the bottom of the homepage. You can also use the same form if you no longer wish to receive this newsletter – just select the unsubscribe button.

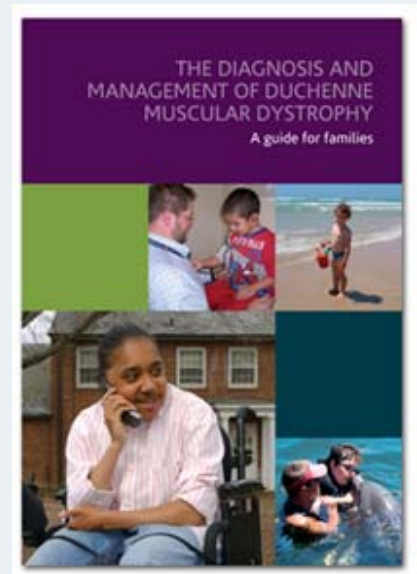
Working with us

TREAT-NMD aims to be an inclusive rather than an exclusive network, and you do not have to be based in Europe or be a partner to be involved. International collaboration with experts from all over the world is already taking place, and new links are being developed.

If you are involved in any of TREAT-NMD's areas of interest and have something you'd like to say or a suggestion of where we could work together, we encourage you to get in touch by writing to us at info@treat-nmd.eu. The coordination team in Newcastle will be happy to put you in touch with the person most relevant to your particular interest.

Family guide for Duchenne Muscular Dystrophy available in several languages

The family guide on DMD called 'The Diagnosis and Management of Duchenne Muscular Dystrophy' is the result of an international consensus on the medical care of Duchenne Muscular Dystrophy (DMD). This effort was supported by the US Centers for Disease Control and Prevention (CDC), in collaboration with patient organisations and the TREAT-NMD network. The recommendations are based on an extensive study by 84 international experts in DMD diagnosis and care. The family guide will provide you with basic information to allow you to participate effectively in a good management of DMD. The family guide can be used in two different ways. Some families prefer to concentrate on the stage of DMD that their child has reached. Others like to understand as much as possible about every aspect of DMD from the start. You can find the Family guide for Duchenne Muscular Dystrophy at: <http://www.treat-nmd.eu/patients/DMD/familyguide> The guide already exists in English and will soon be available in several languages including Bulgarian, Czech, Dutch, German, Greek, Japanese, Portuguese, Romanian, Slovenian, Spanish, Turkish and Ukrainian. For a full overview in what languages the Family guide is currently being translated, please look at: <http://www.treat-nmd.eu/patients/DMD/dmd-guide-languages/> You can find the full academic article published in English in the journal Lancet Neurology at: <http://www.treat-nmd.eu/patients/DMD/dmd-care/>



TREAT-NMD would like to have the guide translated in other languages as well. Please contact us when you are willing to translate the guide in your language when it is not available yet.

Available translations on the Standard of Care in Spinal Muscular Atrophy (SMA)

One of the important activities of TREAT-NMD is to develop Standards of Care for DMD and SMA. Receiving the best care can improve the quality of lives and even prolong life expectancy of people with a neuromuscular disease. Up till now there was not much consensus among physicians about what the best care for people with a neuromuscular disease is. It didn't only differ from country to country, but also within countries. For DMD the academic article published in the journal Lancet Neurology and the Family guide on 'The Diagnosis and Management of DMD' became recently available. The intermediate recommendations on the Standard of Care for DMD that were written and published on the TREAT-NMD website and that has been translated in several languages already, is still valuable. We only now have also an official scientific publication in Lancet Neurology that shows there is an international consensus on the diagnosis and management on DMD. From this scientific publication the family guide on DMD was written so everyone without a medical background can read and understand the information.

As there was already a full consensus statement for the Standard of Care for Spinal Muscular Atrophy, the SMA précis are still valid. The Standard of Care for SMA has been translated into: English, German, Dutch, Russian, Italian, Polish, Turkish, Czech and Serbian. You can find them at: <http://www.treat-nmd.eu/patients/SMA/sma-care/>

TREAT-NMD needs your support to translate the Standards of Care in your language!

As you can read in the above standing text the translations are available already in several languages. But there are still much more languages in which the Standards of Care should be available. It is about your health and the health of others in your country. Please let us know if you are interested to translate the Standards of Care for DMD (Family guide), SMA or Both! TREAT-NMD is a network and we must work together to let this network of people involved in neuromuscular diseases be a success.

TREAT-NMD Spring School and Training

TREAT-NMD organises training and Summer/Spring Schools in Eastern Europe. This year the European Neuromuscular Centre (ENMC), partner of TREAT-NMD, has organised a Spring School from 17 - 20 May in Saint Petersburg, Russia. At this event physicians from all over Russia



Participants and organisers of the Spring School in Russia.

participated and also it was the first opportunity for the patient organisation to meet and discuss issues with physicians working for neuromuscular diseases. A report will be written on this Spring School and published in the next TREAT-NMD Newsletter.

The European Neuromuscular Centre (ENMC) organises a TREAT-NMD training course in Istanbul on the topic "Translating research into clinical practice in Neuromuscular Disorders". This training will take place from 2 - 3 July 2010. On this training too a report will be written and published in the next TREAT-NMD Newsletter.

WANDA (World Alliance of Neuromuscular Diseases Association) Session at the 12th International Congress of Neuromuscular Diseases



During the 12th International Congress on Neuromuscular Diseases (ICNMD) in Naples (Italy) the WANDA will have a separate session at 21 July 2010. For more information on the WANDA program and for registration, please look at: <http://www.icnmd2010naples.org/p-wanda.asp>

TREAT-NMD - Serving the neuromuscular community



Recent years have seen rapid developments in the neuromuscular field and a corresponding surge in interest from the pharmaceutical industry. Promising preclinical results raise the potential for new therapies in the near future, but still some barriers need to be faced before these potential therapies really can be used by patients. Also the lack of standardized care guidelines prevent many people with a neuromuscular disorder to receive optimal care. For the biomedical industry it has been a challenge to find investigators and research sites with expertise to work with. Clinicians and researchers from their side lack supportive tools such as validated clinical outcome measures (tests to decide whether a treatment being tested in a clinical trial is having any effect) or standard operating procedures for research protocols. This lack holds back the therapeutic development.

TREAT-NMD addresses all these issues, uniting the stakeholders in the community and providing an infrastructure that is accelerating research and therapy development, increasing collaboration, improving patient care and helping to support 'clinical trial readiness' on an international scale.

TREAT-NMD was established as a EU-funded 'network of excellence' with the remit of 'reshaping the research environment' in the neuromuscular field. The network has become a global organisation bringing together leading specialists, patient groups and industry representatives towards therapy development in the near future.

To give you an insight on what kind of topics, subjects and issues TREAT-NMD is working on, TREAT-NMD created a brochure with a good overview on this. You can read or download this brochure at: http://www.treat-nmd.eu/userfiles/file/TREAT-NMD_brochure.pdf

Report on the International Conference in Brussels on "Bringing down the barriers in translational medicine for inherited neuromuscular diseases"

The conference in Brussels held from 17 - 19 November 2009 was very successful. 350 delegates from over 30 countries attended and contributed. During the conference several discussions were held in the various sessions. It really encouraged genuine debate between delegates that continued long after each session ended. Dr. Günter Scheuerbrandt, a biochemist from Germany, has written a comprehensive report on this conference that now is available to download. It will not explain all scientific details or contain all discussions and interactions, but it tries to inform you as good as possible on that what has been discussed during these days. The report is available in English, Spanish and German.



One of the interactive sessions at the conference.

You can find the report at: <http://www.treat-nmd.eu/about/treat-nmd-conference/introduction/>

CARE-NMD project for Duchenne muscular dystrophy funded under the EU's Second Programme of Community Action in the Field of Health with one million Euros

The CARE-NMD project is led by the University Medical Center Freiburg in Germany and will help to improve treatment for Duchenne muscular dystrophy patients throughout Europe



Duchenne muscular dystrophy or DMD is the most common of the muscular dystrophies, affecting approximately 1 in every 3,500 newborn boys. As Nick Catlin, father of a son with DMD explains:

"DMD is a heartbreaking disease. Children begin to see their muscles waste away and families struggle to cope with the diagnosis and day-to-day management of this condition. Young men with DMD die early, in their late teens, or are left wheelchair-bound and unable to move unaided"

Although centres for the care of patients with neuromuscular disorders do exist in most European countries, many patients still do not receive treatment according to current guidelines and recommendations. This is

particularly the case in Eastern Europe, where lack of information and lack of access to diagnostic and care expertise creates particular inequalities resulting in reduced life quality and life expectancy for DMD patients.



Participants at the official launch of the CARE-NMD project in Luxembourg

CARE-NMD (Dissemination and Implementation of the Standards of Care for Duchenne Muscular Dystrophy in Europe) was selected for funding by the Executive Agency for Health and Consumers (EAHC) out of 257 applications. The project spans Europe, with partner institutions and patient advocacy groups in

Bulgaria, Denmark, Germany, United Kingdom, Poland, Hungary and Czech Republic (funded partners), as well as Croatia, France, Ireland, Macedonia, Netherlands, Belarus, Romania, Russia, Serbia, Slovakia, Sweden and Ukraine (collaborating partners).

Under the leadership of Dr. Janbernd Kirschner (Department of Neuropediatrics and Muscle Disorders at Freiburg University Medical Center, director Prof. Rudolf Korinthenberg), existing treatment standards and availability in these countries will be evaluated and improved through specific training sessions and other measures. "With a secured diagnosis and adequate treatment, children with DMD and their families can be helped to a far higher life expectancy and improved quality of life." explained Professor Kate Bushby of Newcastle University, United Kingdom. "Better availability of specialist care will also benefit patients with other forms of neuromuscular disease across Europe.

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For more detailed information about the project see www.care-nmd.eu

The Value+ Toolkit, For Patient Organisations On Meaningful Patient Involvement - Patients Adding Value To Policy, Projects And Services



This toolkit for patients and patient organisations has been prepared as a result of the Value + project, which addressed the growing need realised by the European Patients' Forum (EPF) and the European Commission (EC) to support the exchange of information and experience on good practice relating to patient involvement in EC projects. EPF is the umbrella organisation of pan-European patient organisations active in the field of European public health and health advocacy.

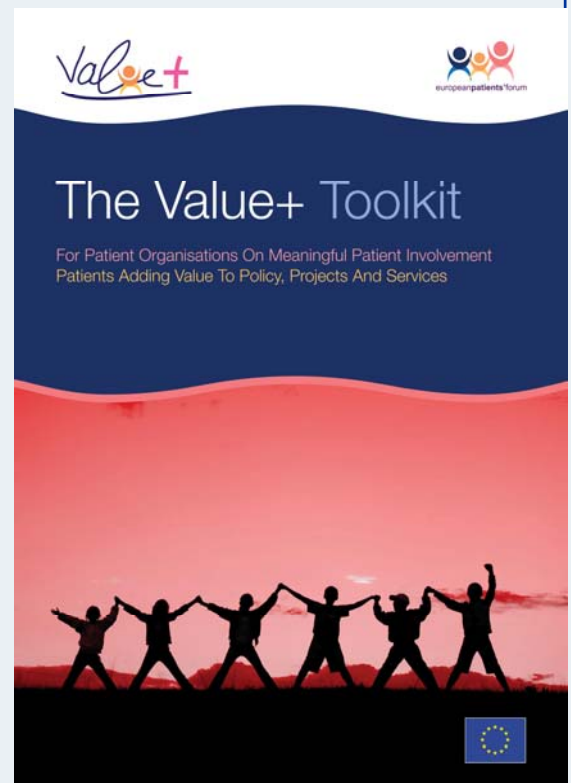
Patient involvement itself is generally taken to mean that patients take an active role in activities or decisions that will have consequences for the patient community, because of their specific knowledge and relevant experience as patients. A unique feature of the Value + project is that patient involvement has been researched and evaluated by patients themselves, rather than by academics.

This toolkit has been written for patient organisations, especially those who want to apply for funding for their own projects, or work with other partners on a proposal for EC funding. Some of the topics in this toolkit will also be useful to project co-ordinators and others responsible for organising patient involvement.

The main purposes of this toolkit are to:

- Support patient organisations in becoming involved in a meaningful way, particularly in EC-funded projects as project leaders or partners. This may mean increasing the skills within the organisation so that the organisation can expand its activities and build its capacity.
- Give information about good practice in patient involvement.
- Provide a definition of 'meaningful patient involvement'.
- Provide a model for meaningful patient involvement in projects, and a way to evaluate involvement according to the model.
- Support the planning of patient involvement from the beginning of a project of activity.
- Support the evaluation of the quality and impact of patient involvement.
- Explain why certain issues are more important to patients, so that project co-ordinators, health professionals and so on are more likely to plan patient involvement round these issues.

You can download a copy from the European Patients' Forum website: <http://www.eu-patient.eu/Initiatives-Policy/Projects/ValuePlus/Resources/Value-Resources/>



Participation of patient groups in TREAT-NMD network

Your help needed by reviewing Toolkit on 'How to set up Expert Centres'

During discussions with several patient organisations and patient representatives the European Neuromuscular Centre (ENMC) noticed that in some countries no expert centres are available, while at the same time patients do feel a need for it.



Front page of a brochure of a Centre for Neuromuscular Diseases in the UK.

What can you do as a patient organisation to stimulate the set up of an expert centre! In some countries it takes quite some persistence to get in contact with physicians and scientists and then one also must hope to be taken seriously. In the past patient organisations were seen as groups of people with a disease who were not really able to contribute to science and medical development. The days have changed however, and right now patient organisations are groups of people who know very well what they want, and who do have knowledge and experience on their diseases. This knowledge and expertise of patient organisations must not be thrown away and must be used.

But what can you do when you need good medical care and treatment and just a few medical specialists in your country are interested in your disease! In the toolkit 'How to set up an expert centre' the ENMC aims to support patient organisations to set up such an expert centre in the several countries. However to create such a toolkit it is good to get the input and feedback from you as well. Please let us know if you are interested to review the toolkit we are working on now. Any comment and input is welcome. If you are interested, please contact us at: schoneveldvanderlinde@enmc.org After your email, we will send you the draft version of the toolkit for your review.

Patient Voice Working Group TREAT-NMD Workshop

At 15 - 16 September 2010 the PVWG TREAT-NMD Workshop will be held in Milan, Italy. The workshop is upon invitation only to make sure all countries at least can be represented by one patient organisation or patient representative. The invitations will be send out soon. We encourage the invited patient organisations and representatives to get in contact with the colleague patient organisations in ones country and to work together on this.

Send us your news and views!

We strongly encourage everyone to become member of TREAT-NMD and to send their own news and updates and we will be happy to include them in future editions of the regular digital newsletter. Please send your contributions to Maryze Schoneveld van der Linde at: schoneveldvanderlinde@enmc.org

