

welcome

Welcome to the 76th Newsletter from TREAT-NMD.

This week's newsletter includes...

- an update on the Patient Voice Working Group Meeting in Denmark
- a focus article on UPA!
- a Question & Answer session regarding patient registries
- details of the Paris and Berlin myology summer schools

We would like to thank those who have contributed to this week's edition. This newsletter relies on input from our readers. If you have anything you wish to be included in the next newsletter please contact us at info@treat-nmd.eu

at a glance...

[2-7 May 2010 International Child Neurology Congress 2010 - Cairo, Egypt](#)

[10 May 2010 The George Karpati Symposium on Neuromuscular Disease: Innovation and Application, Montreal, Canada](#)

[12-15 Jun 2010 European Human Genetics Conference - Gothenburg, Sweden](#)

[13-15 May 2010 5th European Conference on Rare Diseases - Krakow, Poland](#)

[17-19 Jun 2010 International Conference on Neuromuscular Diseases - Sao Paulo, Brazil](#)

[19-23 Jun 2010 20th Meeting of the European Neurological Society - Berlin, Germany](#)

[24-27 Jun 2010 Parent Project Muscular Dystrophy Annual Conference - Denver, Colorado, USA](#)

[24-27 Jun 2010 Families of SMA Annual Conference - Santa Clara, California, USA](#)



Patient Registries - Conference Question & Answer Session

The TREAT-NMD / NIH conference held in Brussels last year enabled participants to submit questions prior to the start of the conference for panels of experts to deliberate over and respond to during the conference itself. Due to time constraints it was not possible to answer all questions addressed to the patient registries during the conference and so a Q&A document has been drafted by discussants of the registry session to provide answers to all questions submitted.



It must be noted however that, the statements reflect the opinion of the contributors only. They should not be regarded as official statements of the NIH or TREAT-NMD.

The first question is included below. If you would like to see the whole document please click [here](#).

Q: How should questions of data ownership in clinical research be resolved? To what extent do patients and their families own the data they contribute to researchers, sponsors, and regulatory authorities? What model of data ownership would patients and their organisations see developed?

A: Data contributed by patients and their families belong to the individual national patient registry. Personal and medical data will be kept for an indefinite period under the responsibility of the Principal Investigator of the national registry. These data are subject to the regulations on data protection (national laws related to EU directive 95/46) and all information received from patients will be treated confidentially. Patient participation is, however, voluntary; patients may decline to participate or withdraw consent for their data to be stored on the registry at any time without prejudice. In this respect individual patients do own their own data; they can decide to contribute to the patient registry or to withdraw their personal and medical data at any time of the existence of the patient registry. For patients, more important than ownership in a legal sense is: What happens to their data and who has control over their data? These questions are addressed in the Registry Charter; for example, the patients' right to withdraw their data at any time. Furthermore, patients/parents are asked to consent to being on the registry. In the informed consent form, it is explicitly stated that de-identified data will be shared with the TREAT-NMD Global Patient Registry and that de-identified data may be further shared with others beyond TREAT-NMD.

Third parties wishing to have access to data in the TREAT-NMD global registry, such as researchers or companies planning clinical trials or conducting research on new therapies, will only have access to anonymous information identifiable only by a code. Before they are granted access even to this anonymous information, they will have to have the approval of an Ethics Committee and the TREATNMD Global Database Oversight Committee (TGDOC). Patient data will not be made available to employers, governmental organizations, insurance companies, and educational institutions, or to a patient's family member or doctor.

Data used by researchers and published in journals or other scientific publications are owned by the researchers themselves. As soon as anonymised medical data of patients is processed and analyzed, the owner is the researcher. It is not possible to withdraw your medical data from already published material.

In summary, ownership of data is variable depending on 'the level' at which the data is stored or is being used:

Level 1: Patient is owner of own individual medical data and is free to contribute them to the patient registry and to withdraw them at any time during the existence of the registry.

Level 2: The collected data of patients is maintained by the institution of the national patient registry. Even when the data of a patient are collected and stored in the patient registry, a patient can always withdraw his/her own personal and medical data.

[19-23 Jul 2010 12th International Congress on Neuromuscular Diseases - Naples, Italy](#)

[18 Sept 2010 Muscular Dystrophy Campaign Conference - Birmingham, UK](#)

[12-16 Oct 2010 World Muscle Society International Congress - Kumamoto, Japan](#)

[12-13 Nov 2010 Action Duchenne 8th Annual Conference - London, UK](#)

Please note: This is only a selection of upcoming meetings. To see all our listed meetings [click here](#).

Level 3: The processed, analyzed or published material coming from a patient registry is owned by the principal investigator of the research, or author. It is not possible for an individual patient to withdraw his/her de-identified data when their data is already being used or in a publication. It is possible, however, for an individual patient to withdraw the data for future use.

This is only the first question if you would like to see the whole document please [click here](#).

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Focus on UPA! Latin/Ibero-American Network

TREAT-NMD has close working relationships with patient groups across the world and in this newsletter, in the first of a series of "focus on" articles, we are featuring the work of UPA!, a Latin and Ibero-American patient advocacy group.



In 2007, Leslie Guzman established UPA! cura Duchenne with the purpose of integrating under one emblem and name the diverse efforts of leading Latin and Ibero-American organizations focused on treatment, therapies, and a cure for Duchenne and other neuromuscular disorders.

UPA!'s main objective is to give children and young people with Duchenne/Becker, along with other people affected by neuromuscular disorders, the opportunity to live a normal life, and to work towards treatments for the current generation of children and young people suffering from neuromuscular diseases. UPA! is dedicated to improving the quality of treatment for patients by encouraging Ibero-American associations with common goals to participate together in global efforts, seeking unity and collaboration between associations, integrating the region, and gathering knowledge and resources in an attempt to heighten the understanding and consciousness of society and government regarding neuromuscular disorders.

What we do for our region

We function as an Information Center of Excellence in Spanish in which the Spanish speaking population can find answers to their questions about Duchenne, its progression, treatment, care, therapy, genetic counseling, research and studies, among other relevant themes. We do this by translating and publishing information through newsletters and on our website, running the www.upaduchenne.org blog and social networks and organizing and taking part in conferences and workshops.

We are currently the point of unity between Associations in Latin America in order to develop joint programs that help the cause. This achievement has allowed us to form the UPA! Iberoamerican Network where other associations located in countries such as Argentina, Ecuador, Peru, Spain and Venezuela can reach us and work together towards our common goals.

Additionally, through our website, it is our aim to help the community by providing relevant information that improves the quality of life of the families affected by DMD, such as current news on advances in existing studies, genetic laboratories, education topics, and much more.

Alliances and International Agreements

Being aware of the impact of uniting efforts to achieve common goals, the first actions of UPA! were focused on identifying and establishing links with the most important organizations in the field. As a result, in 2007, during Debra Miller's visit to Mexico, UPA! signed a strategic alliance with Cure Duchenne, with the goal of facilitating and sharing resources to increase awareness, disseminating information, and fundraising. A press release was issued to publish this event.

At the same time UPA! established collaborative links with Parent Project Muscular Dystrophy, sharing Pat Furlong's efforts in the never-ending battle to remain on the vanguard of advances in research and treatment to end Duchenne and offering most reliable and updated information to the Duchenne community. The UPA! web site is now the best place to find information on Duchenne in Spanish.

Elizabeth Vroom, President of United Parent Project Muscular Dystrophy, contributed with her inspiration in the establishment of UPA!, and we have open support links with UPPMD.

Since 2008 UPA! has been a proud member of the TREAT-NMD network with whom we work closely in our quest to improve the life of many young warriors who every day face new challenges with strength and love.

UPA! México

Supporting Leslie's vision, in 2008 Diego's family members, led by Guadalupe Franco, Diego's grandmother, founded UPA! México as a non-profit association, pursuing the same objectives as the UPA! Network. Following the same model, in Mexico we have established a network of connections with organizations, public and private institutions, research centers, health professionals and government offices for their support to our cause, Duchenne.

Member of the TREAT-NMD network

As a member of TREAT-NMD, UPA! is entrusted with the goal of uniting efforts in order to collaborate and communicate openly to support neuromuscular projects. Through Katie Bushby we have followed the advances of this Network of Excellence and have taken part in joint efforts.

UPA! is working with TREAT-NMD on their efforts to have a global database of patients with Duchenne and other neuromuscular disorders, seeking to compile data for further research and developments. To join this effort, we have implemented a Latin-Iberoamerican Registry for Duchenne, serving the Spanish-speaking community. The UPA! Registry is now being adopted by several countries in Latin America as their national registry. The UPA! registry can be accessed via our website <http://www.curaupaduchenne.org/registro/index.php>; it is currently being filled out by members of UPA!

and will continue to evolve dynamically.

UPA! is also collaborating with TREAT-NMD and other organisations on translating information material into Spanish. A Spanish version of the valuable *Lancet Neurology* publication on the Diagnosis and Management of Duchenne muscular dystrophy will be available soon, as will the related Family Guide for Duchenne, a most helpful document for our families to have. UPA! is also aiming to print the Family Guide in brochure format and make it available to as many families as possible throughout our region.

On behalf of our community members, UPA! would like to state our open recognition and gratitude to all people and organizations that are working hard to change the lives of all our children and young men with Duchenne and their families. Our thanks to you all.

If you would like to know more about us and join UPA!'s efforts please visit our website www.upaduchenne.org or contact us: [Leslie Guzman](#) or [Guadalupe Franco, PhD](#)

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Patients' and Patient Organisations' Involvement in TREAT-NMD Workshop - Postponed

Due to the unforeseen travel disruption caused by the Icelandic volcano eruption, the organising committee of the Patient Voice Workshop, Patients' and Patient Organisations' Involvement in TREAT-NMD, scheduled to take place in Musholm Bugt Feriecenter, Denmark from 23 – 25 of April 2010, decided that it was necessary to cancel the planned workshop.



Understandably, it was a difficult decision and the organizing committee would like to thank the meeting participants for their constructive input, which enabled them to make the final decision.

Fortunately, the organizing committee was immediately able to offer a new date and venue for a re-scheduled workshop. We are pleased to announce that the workshop, with a similar format to the one previously planned, will take place 15-16 September 2010, immediately prior to the EAMDA meeting, in Milan, Italy.

In the meantime, TREAT-NMD will be working on establishing a web page dedicated to the Patient Voice. The webpage will include the possibility for patient and patient advocacy groups to submit questions and suggestions to the Patient Voice Working Group. We would welcome your suggestions, in this early planning phase, and would invite you to submit them directly to [Maryze](#) or [Michael](#).

In addition, we believe that the WAMDA meeting to be held in Naples will also begin to address some of the topics identified for discussion during the patient voice workshop and should feed into the meeting in Milan.

Further details regarding the Patient Voice Page and updates on the meetings planned for Naples (WAMDA) and Milan (PVWG and EAMDA) will follow in future newsletters.

On behalf of the Patient Voice Workshop Organizing Committee

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MyoGrad muscle science summer school in Berlin 14-18 Jun 2010

In a new initiative linked to the longstanding summer school of myology held every year by the Institut de Myologie in Paris, MyoGrad now invites young scientists and physicians to Berlin for a related course in muscle science.



The Summer School for Myology Paris-Berlin spans topics ranging from basic muscle science to clinical myology. Two teaching modules, one in Berlin and one in Paris, comprise more than 60 lectures held by world-leading scientists and clinicians who can provide expert knowledge on this highly specialized subject.

The Muscle Science Summer School in Berlin will cover the whole field of basic muscle research, with lectures on muscle contraction, muscle development, muscle metabolism, signaling pathways and novel experimental treatment strategies.

The course will take place from 14-18 Jun 2010 and the deadline for applications is

15 May 2010

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