

WORLD ALLIANCE OF NEUROMUSCULAR DISORDER ASSOCIATIONS

WANDA Update July 2003

To: all Muscular Dystrophy / Neuromuscular Disorder Associations (NMDAs)

Subject: update of WANDA activities

WANDA/Vancouver

After the WANDA- meetings in Vancouver and the impressive quadrennial IC- NMD successful congress the board of WANDA is further structuring and developing its policy within its priorities:

* to encourage the formation of associations for neuromuscular diseases in countries without such existing bodies by providing information, advise and support.

* to develop on the international level strategies to shorten the road from diagnosis to therapy.

The latter objective is currently worked out with the Gaetano Conte Academy in Naples with a focus on epidemiological studies.

WANDA/Istanbul

The next IC-NMD will take place in the first week of July 2006 in Istanbul . WANDA intends as usual to organize its 5th general assembly in conjunction.

WCAF and New Website

The WANDA Conte Academy Forum (WCAF) is a joint venture between the Gaetano Conte Academy, in Naples, and WANDA. The aims of WCAF are:

- a) To serve as a forum to facilitate
 - dialogue and the exchange and dissemination of information about neuromuscular disorders including, but not limited to, scientific, clinical and psycho social topics and to facilitate
 - policy making and strategic planning particularly in regard to the development of effective therapeutic approaches in neuromuscular disease
- b) To promote the cooperation between organisations working directly or indirectly for the benefit of people involved in neuromuscular disease
- c) To contribute to the education and/or empowerment of people with a neuromuscular disease with an emphasis on people in developing countries.

To achieve these goals WCAF organises workshops and will maintain a website.

Objectives are:

- a) To create a reliable link between families, parent/patient organisations, doctors and scientists following a specific policy program.
- b) To promote existing activities and strengthen new initiatives within the framework of the policy program.
- c) To function as an instrument for education, public awareness and empowerment.
- d) To initiate policy making and strategic planning particularly in regard to the development of effective therapeutic approaches in NMD.
- e) To catalise areas of specific interest such as in the field of epidemiology and translational research.

This new web site will go a long way in providing accurate, relevant and reliable information to help satiate the enormous demand on information on neuromuscular disorders. It will also

provide easy links to all neuromuscular disorder associations. It will promote activities of WMS, MSM and ENMC..

Development of the site will take place in three phases.

It is expected that stage one of the web site will be complete and online by the end of August 2003. Information will be circulated to all WANDA members as soon as the site is online.

WCAF and New Approaches in Epidemiology

“New Approaches in Epidemiology in Neuromuscular Disorders” was the title of a workshop held in conjunction with the MSM conference in Corfu. This workshop focused on new approaches to this subject and was organised by the WANDA Conte Academy Forum (WCAF). This workshop is part of WCAF’s strategy and policy development for research into the causes and treatment of, and new medicines for, neuromuscular disorders. It is also part of our joint strategy of facilitating epidemiological studies. The full report on the workshop was published in ACTA MYOLOGICA Volume XXI, December 2002 p151-153.

Trials and ENMC

Prof. Richard Hughes and dr. Michael Rose of the Department of Clinical Neurosciences of the Guy’s King’s and St. Thomas’ School of Medicine in King’s College London, have been appointed to mediate in the development of systematic reviews and preparation of applications for clinical trials. They bring with them support from the Cochrane Neuromuscular Disease Systematic Review Group, which is based in their department. Over the next three years this team will invite all those involved with the ENMC to take part in the effort to provide the evidence base for the management of people with neuromuscular diseases. This will involve not just medical and paramedical staff, but also patients and European patient groups. We have broken down the tasks into the following groups.

- Identification of systematic reviews needed
- Recruitment of authors and registration of titles with the Cochrane Collaboration
- Publication of the protocols for systematic reviews
- Publication of systematic reviews
- Identification of key trials and their leaders
- Workshops for key trials
- Submission of grant applications for key trials
- Disease registers for monitoring treatment
- Training courses in assessing outcome measures and undertaking clinical trials.

Quoting from the ENMC- website (www.enmc.org): “We need to work with European investigators, patient support organisations and grant-giving bodies at national and European levels to obtain the funding for the trials. Throughout the process we will be consulting basic and clinical scientists working on the mechanisms and potential treatments for neuromuscular diseases. This initiative needs the enthusiastic collaboration of as many as possible of the clinicians, scientists and people involved with neuromuscular diseases. If you want to write a systematic review, consult the Cochrane Neuromuscular Disease website (<http://www.kcl.ac.uk/cochranenmd/>) and contact Kate Jewitt, the co-ordinator, at kate.jewitt@kcl.ac.uk. If you want to mount a clinical trial, contact Professor Richard Hughes via the ENMC office in Baarn at enmc@enmc.org.

Therapy for Pompe disease

WANDA’s first focus disease

Pompe disease, acid maltase deficiency, was WANDA's first focus disease. (see extensive report in WANDA- 2000 newsbulletin). During various WANDA- conferences special sessions were organised to focus attention for this disease.

The International Pompe Association, representing patient groups in many countries all over the world, work closely together with the pharmaceutical company Genzyme on the development of a treatment for Pompe disease by enzyme replacement. As part of these efforts, Genzyme and the International Pompe Association jointly publish regular updates on the status of Genzyme's program. We invite you to read the latest update from which we quote the following text.

Clinical trials

Genzyme continues to make progress within its program to develop a treatment for Pompe disease. The company is currently conducting clinical trials evaluating the safety and efficacy of enzyme replacement therapy with recombinant human acid alpha-glucosidase (rhGAA).

In March 2003, Genzyme began enrolling patients in a clinical trial including 16 children between the ages of six months and three years with the infantile-onset form of Pompe disease, in whom symptoms presented during the first year of life. It is scheduled to last one year. Detailed information about the trial's entry criteria and protocol can be found at <http://clinicaltrials.gov>. At present, enrollment is open at five research hospitals (Duke University Medical Center, Cincinnati Children's Hospital Medical Center, and the University of Florida at Gainesville in the United States, the Royal Manchester Children's Hospital in the United Kingdom, and Hôpital Universitaire Debrousse in Lyon, France).

Genzyme intends to seek product registrations globally.

Expanded access/compassionate use

Genzyme has received requests for rhGAA for patients who do not qualify for studies 1602 or 1702. A senior management committee within Genzyme closely monitors material inventory, and should material become available, Genzyme hopes to explore making it available on an expanded access basis. Any process for doing so will need to be conducted in coordination with regulatory authorities. At the moment, however, Genzyme must focus on using all available enzyme to conduct the clinical studies that it hopes will demonstrate that rhGAA is efficacious and safe, so that it can apply for approval for medical use as soon as possible.

Pompe disease website

Genzyme is pleased to announce the launch of www.pompe.com, a website dedicated to providing people with Pompe disease, their families, physicians and the entire Pompe Community with comprehensive information on Pompe Disease. Visitors to www.pompe.com can find detailed information on a broad array of important topics, as well as a list of resources available for help in addressing many aspects of the disease. The site was created with input from people living with the disease and physicians throughout the community.

For more details please contact Dr. Ria Broekgaarden, secretary International Pompe Association (IPA), VSN, The Netherlands; e-mail: ria.broekgaarden@vsn.nl

Therapy for SMA

SMA is WANDA's second focus disease and in this context several international workshops were organized through WCAF: Capri (focus on genetics), Corfu (focus on epidemiology) and through WANDA: Vancouver (focus on translational research). A strategy has been developed for and with family groups in Europe who founded a European Taskforce focusing on getting research subsidies from European Union resources for patient registries, trial protocols and reviews. These European family groups organized meetings in Amsterdam (by Dutch NMDA), London (by JTSMA) and Paris (by French NMDA). Last month families of SMA (FSMA) organized a congress in Washington (for report see www.fsma.org).

Myoblast Transfer Treatment

All associations should have received the WANDA circular letter about myoblast transfer treatment during the past week or so. (Contact the Secretary General if you have not received a copy). Further information on this topic can be found at www.cardiomiologia.it then click on MSM. WANDA is interested to be informed about further experiences of families.

WANDA Development

Since the very successful Fourth WANDA Assembly held in Vancouver last year, WANDA has been reflecting on its continental activities and addressing the challenge of activating strategies for developing effective ways by which we can learn from each other. There are great differences between continents which in itself, provides great challenges. Within these continental approaches we have been working in cooperation with ENMC, WMS and also WHO, UNESCO and the Global Life Sciences Forum

Global Life Sciences Forum

The Congress of the Global Life Sciences Forum took place in Lyon, France in April 2003. Our vice president for Europe, Ysbrand Poortman, was one of the chairpersons for this multidisciplinary conference. Attended by over 20 Nobel Prize winners, formally opened by the French President, Chirac, this was indeed a prestigious meeting. Sebastian Kesler of the Swiss NMDA also attended this congress. A more detailed report from Mr Poortman on the aspects of the Congress relevant to neuromuscular disorders will appear in our next News Bulletin.

WANDA Website

The WANDA website is about to be upgraded. Work will begin within the next few weeks with completion expected within a few months. Any suggestions or inclusions for the revamped website will be most welcome.

Conferences on NMD

We have received many reports of congresses and conferences on neuromuscular disorders and will post this on our website as soon as available.

One example was a very successful Muscular Dystrophy conference held in Perth, Western Australia, over the 9th and 10th May 2003. Delegates came from all states of Australia and internationally from Singapore, Korea and New Zealand. Speakers came from as far away as Great Britain and the USA.

WANDA contacts

Should you wish to contact WANDA please address your correspondence to your region's vice president or the Secretary General.

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