

**MEETING REPORT**  
**Extended Network of nEUroped**  
Fondation Universitaire  
Brussels, Belgium  
November 23<sup>rd</sup> 2010, 9.00am-15.30 pm

The meeting of the **Extended nEUroped Network** was held within the *nEUroped* project event that took place on 21, 22 and 23 November 2010 at the Fondation Universitaire in Brussels, Belgium.

**Specific objectives**

Within the overall objectives of the project meeting to bring together all partners of nEUroped and potentially interested parties and to examine the work and the achievements of this three year project, the specific objectives of the Extended Network of nEUroped day were to:

- ✓ Review the work accomplished in the nEUroped project in particular on the development and validation of disease guidelines
- ✓ Develop the future of the nEUroped Consortium: extend the Network to members outside the project and to patient networks.

**Program**

The meeting was structured in three sessions:

1. Disease Guidelines for nEUroped with Chairpersons Rosa Peraita Adrados, University Hospital Gregorio Marañón (UGM), nEUroped, Spain and Brian Neville, Institute for Child Health, University College London, nEUroped, United Kingdom
2. Integrating Disease and Best Practice Guidelines into European Patient Registries, Chaired by Claudio Zucca, IRCCS "Eugenio Medea", nEUroped, Italy
3. Future Perspectives on the nEUroped Networks' Organisation and Sustainability, with Chairpersons Tsveta Schyns, European Network for Research on Alternating Hemiplegia (ENRAH), nEUroped, Belgium and Francis P. Crawley, Good Clinical Practice Alliance – Europe (GCPA), nEUroped, Belgium.

**Achievements**

**1. Diseases Guidelines**

In his introduction to Developing Guidelines for Patient Care in nEUroped Prof. Brian Neville summarized the method applied to this group of rare diseases that includes:

- Creation of patient registries
- Increasing awareness of professionals, carers and patients
- Developing of services and Service provision
- Facilitation of research directed to disease's pathogenesis and treatment and to insight into rare disorders generally

Guidelines for diagnosis, treatment and management of rare paediatric neurological disorders with paroxysmal attacks are major aim of the nEUroped project. The process of developing of Guidelines includes 3 separate stages: Academic review of evidence, Guidelines for parents and professionals and Lay person's explanatory booklet. The At the first stage, an academic review of published evidence on diagnosis, treatment and management of the respective diseases under nEUroped should be developed to reflect

the achieved consensus among the professionals and parents/patients involved. These documents should be published and, in order to be developed as guidelines, should be reviewed every 2-3 years and updated with latest research results. General problems encountered are the lack of data and resources.

Assessing and integrating into the Guidelines the real life needs of children with rare paediatric neurological disorders with paroxysmal attacks is a further challenge as all aspects, medical, educational, behaviour treatment and social services are usually taken care separately (e.g. paediatricians are variably trained in behavioural disorders, child psychiatrists are largely not trained in paediatrics or neuro-disability etc). Nevertheless, these First Drafts of Guidelines for diagnosis, treatment and management in these rare paediatric conditions will be of benefit by setting up auditable standards and by indicating the research needs (and thus, serve as reference for research funding efforts) . The Guidelines can be powerful if adopted by national and international organizations and can be used for advocacy and for strengthening support groups.

The Draft Proposal for Guidelines for diagnosis, treatment and management of Narcolepsy in children was presented by Prof. Sona Nevsimalova nEUroped Working Group Leader in Narcolepsy, Neurological Clinic of 1st Medical Faculty of Charles University, Czech Republic. The following principal needs of children with Narcolepsy are reflected in the Draft proposal: Timely /early diagnosis; Adequate treatment (medication and non-pharmacological procedures) that is supported by clinical trials; Support in all areas – education, psychotherapy; Access to information about the disease for parents, other family members and school teachers (TV, radio, booklets, videos...). By reviewing the current diagnostic methods and therapeutic interventions available for children with Narcolepsy, the Working Group has drawn several areas where nEUroped can particularly contribute in the future : scientific → by cooperation with European Sleep Research Society (ESRS), the EU Narcolepsy Network (EU-NN), the International Pediatric Sleep Association (IPSA); pharmaceutical → by initiating clinical trials in children with Narcolepsy with new or already existing drugs; education → to improve public knowledge about the disease with help of the patients' organisations (e.g. European Narcolepsy Day – every year, March 18); social → incorporation in school education and employment, help in requalification.

## ***2. Integrating Disease and Best Practice Guidelines into European Patient Registries- the need for Health Indicators in the registries for rare diseases***

The presentation of Dr Laura Fregonese from the European Union Committee of Experts on Rare Diseases (EUCERD) and the follow up discussion emphasized:

- the applicability of the Patient Registries as an efficient and consistent reporting mechanism to measure RD globally and individually as a public health issue and to enable surveillance of status and trends
- the role of patient registries and the participating centres and clinicians is extremely important for the production of good quality of care indicators, in particular in the field of rare diseases

and concluded that health indicators are of particular relevance for rare diseases and should be included in patient registries and guidelines. These should include mortality age, survival rate from diagnosis, duration from first symptoms to diagnosis, related morbidity, and health expectancies.

### **3. *Ad Hoc Group Discussion on the ENM and nEUroped***

On the request of the Patient Networks, a Roundtable discussion on the project's achievements and challenges took place after the Guidelines session. The discussion was centered around the following questions:

- how can nEUroped contribute to enhance knowledge and facilitate research on rare neurological diseases in children across specialized centers in Europe? – it has been emphasized by both professionals and parents that they expect to be involved and continuously informed on the progress of nEUroped.
- how can nEUroped better engage patients and parents?- parents groups who participate in nEUroped expect that they are equal partners in decision making process concerning the usage of the Registry data.

### **4. *nEUroped future steps: interaction and collaboration with others and sustainability issues***

Opportunities for nEUroped to extend collaborate through the established European Paediatric Network at the European Medicines Agency, EnprEMA, were presented by Dr Ralph Bax from the European Medicines Agency London. He stated that the following recognition criteria to join EnprEMA were:

- Research experience and ability
- Network organisation and processes
- Scientific competencies and capacity to provide expert advice
- Quality management
- Training and educational capacity to build competencies
- Public/Patients involvement

These criteria should be implemented in the **nEUroped Strategic Plan** for development.

Prof Brian Neville concluded in the last presentation of the day that the three aims of nEUroped in order to improve and sustain should be: ascertainment, treatment and management of rare disorders. These can be achieved through:

- Implementing Patient's Registries
- Increasing the awareness of the diseases among the professionals, carers and patients
- Developing services
- Facilitation of research on pathogenesis and treatment
- Service provision.

The session ended with discussion on the possible steps in implementing nEUroped beyond the current funding that will expire in 2011. To achieve its aims and further develop to meet the established European standards, nEUroped should grow at both, national levels and at a European level. Both dimensions, national and European, should be explored for future funding of projects.

### **Indicators**

- ✓ Number and representativeness of meeting attendees

Forty participants (thus, approximately 60 % of the 3 days conference attendees) attended the meeting. These were representing nEUroped project Associated and Collaborating Partners, nEUroped Patient Networks and newly affiliated members of the Extended nEUroped Network, as well as two EU project coordinators and representatives of the European Medicines Agency and the European Union Committee of Experts on Rare

Diseases (EUCERD). The invited Speakers from the EU Commission, DG Health and DG Research were not able to attend this day for logistical reasons.

✓ Evaluation and feedback of participants

The opinion of the meeting participants as expressed in the Evaluation on line form can be summarized as “helpful”. For details on the participant’s feedback, please see the Evaluation Summary in the Appendix (*in the compiled Report ENM*).

### **Recommendations**

- on Guidelines for treatment : publish the first drafts developed during the nEUroped project and ensure that a system is in place to collect feedbacks from experts and patients and to update the drafts each year

- on Sustainability :

1) nEUroped registry and Guidelines should include health indicators as mandatory fields for data collection and for analysis and reporting

2) nEUroped Strategic Plan should adopt the already established European standards (eg the evaluation criteria of EnprEMA) and continue to develop to achieve ascertainment, and better treatment and management of rare neurological diseases with paroxysmal episodes.

### **Conclusions**

*“All of these aspects could be of value, but at the moment they are hypothetical”*

*“We need this project in Europe for our children. The patients want this to succeed.”*

*“This is a hugely important project and must be successful. I would ask those involved to put every effort into making it a success. I would like to thank them in advance for their work.”*

( comments from the nEUroped Evaluation Summary Report)