



LSD PATIENT
COLLABORATIVE



REGIONE DEL VENETO



RARE NEUROLOGICAL DISEASES OF CHILDHOOD

“WE TREAT THE CHILD TO TREAT THE ADULT”

*December 2nd 2010
European Parliament, Room J6Q1
Bruxelles, Belgium*

11:00 – 11:25

Opening Address

Mrs. Amalia Sartori, MEP
Prof. Maurizio Scarpa, B4B Foundation
Prof. Mary Baker, EBC President
Delegate Veneto Region – tbc

Moderator: Michael Rogers

11:25 – 11:40 Keynote Lecture

- Brain, rare neurological diseases and European health care systems. Mr. John Bowis, OBE, UK

11:40 – 12:10 Theme 1 – Brain and Rare Diseases: The Patients' Perspective

- Promoting awareness brain diseases in Europe: enabling the dialogue between science and society – Mrs. Mary Baker, MBE – President EFNA
- Living with a person affected by a rare disease: impact on the family and carers – Mrs. Christine Lavery MBE, LSD Patient Collaborative, UK
- Fighting for and together families– Prof. Francois Eyskens, B

12.10 – 12.45 Theme 2 – Brain and Rare Diseases: The Doctors' and Scientists' Perspective

- The burden of diagnosing rare brain diseases; medical and ethical implications – Prof. Frits Wijburg, NL
- Rare neurodegenerative diseases: searching for a therapy – Prof. Tony Futerman, IL
- Health economic considerations in the treatment of rare diseases – Prof. Mike Drummond, UK
- The voice of industry – Dr. Riccardo Palmisano- AssoBiotec,

12.45-13:00 keynote lecture

- The burden of rare neurological diseases of childhood in Europe: what is the future? Prof. Timothy Cox, UK

13.00 – 13.30

Panel Discussion: Can Brain Research Help Our Society?

- Consensus statement on European brain research and rare neurological diseases

13.30 Lunch

Briefing Note

Why participate in the meeting

According to the World Health Organization, 1 out of every 10 people will suffer from a severe neurological disorder during their lifetime. In Europe, therefore, about 50 million citizens are affected, 15 million of whom will be suffering from a lethal neurological rare disease. The social and economic cost to the EU of Neurological Diseases is huge. Alzheimer itself costs \$400 billion per annum.

The diagnosis of rare diseases is difficult due to a lack of knowledge of these conditions in the general medical community. This hinders especially the early-detection of rare diseases affecting children. The management of disease and therapy also represent a social burden. Treatment is limited, to a few conditions, and more than 90% of them do not have a current therapy. Moreover, more than 95% of so called "neurological drugs" do not reach the brain in therapeutic quantities..

Nonetheless, joint efforts from the European Union, private agencies and industrial companies have developed sophisticated tools to speed up diagnosis and therapeutic strategies. Yet, a lot still has to be done, since no effective therapy is available for the brain.

For this reason the Brains for Brain Foundation, together with the European Brain Council and the LSD Patient Collaborative, has organised this meeting to offer to relevant stakeholders a moment of discussion to share views on the current challenges and find new research strategies to improve therapy and quality of life of families and patients affected by rare neurological disorders.

Aim of the meeting

The major aim of the meeting is to acknowledge the growing interest of the EU Commission to neurological problems. In fact, in the FP7 programme, major grants have been awarded to study neurological disorders under the FP6 Programme. For this reason the meeting wishes to demonstrate the unity of intent of Family Associations, Industrial Companies and Scientific Community in stimulating interest in rare diseases as models for the further therapy of adult and more common brain diseases. We think indeed that by studying the neurological disorders of well defined monogenic diseases affecting children we will be able to find key aspects to develop new and unique therapies for adult disorders such as Parkinson and Alzheimer Diseases, Multiple Sclerosis etc.

Furthermore, the close collaboration with the Political Members to identify the unsatisfied needs of the families and the Scientific Community working on rare neurological diseases of childhood is needed to stimulate the European Community to continue to devote funds for research and social assistance.

We believe that this meeting will generate working groups formed by representatives of Medical Professionals, Biotech Companies and Family Associations working together to collaborate with the EU Commission in treating adult disorders by solving children's diseases.

To whom this meeting is addressed

This workshop is addressed to all stakeholders such as Political Delegates of the 27 Member States interested in social, research and health issues, communication and mass media professionals, Representatives of National and International Scientific and Lay Association/Organisation/Foundations working to generate awareness on rare neurological diseases, to identify the needs of affected patients and to catalyze research toward the understanding and the treatment of lethal neurological diseases of childhood.

The Organising Organisations

The Brains For Brain Research Foundation

The Brain for Brain Foundation and Research Consortium was founded in 2007. It is an organisation formed by highly distinguished basic scientists and clinical neurologists dedicated to an improvement in scientific understanding of paediatric neurological rare diseases, in particular the neurodegenerative disorders, and improving their therapy. At the moment over 60 Universities network and globally (12 EU SM, USA Brazil and Australia) and 10 Biotech Companies are actively collaborating with the Brains For Brain Foundation to develop original therapeutic approaches, which will also be applicable to the more common disorders such as Alzheimer's and Parkinson's diseases, brain tumours, and spinal lateral atrophy, for example.

The Brains For Brain Foundation believes in and actively encourages focused and organised international collaboration in order to facilitate the achievement of the necessary scientific research needed to meet the needs of neurological disease and health, a fundamental right of modern society.

The European Brain Council

The European Brain Council (EBC) is a coordinating council formed by European organisations in neurology, neurosurgery, psychiatry, basic brain research (neuroscience), as well as patient organisations and industry. It therefore represents a vast network of patients, doctors and scientists, and these stakeholders along with its industrial partners make it eminently suited to work in close partnership with the European Union commissions, the European Parliament and the World Health Organization (WHO), as well as other decision-making bodies. The EBC was officially founded on 22 March 2002 in Brussels, and has offices in Brussels and Florence.

The LSD Patient Collaborative

The UK LSD Patient Organisation Collaborative represents those affected by Lysosomal Storage Disorders as an action group to work and lobby on behalf of LSD patients and their families in the UK. The Group is made up of representatives from the [Association for Glycogen Storage Disease](#), [The Batten Disease Family Association](#), [The Gauchers Association](#), The Society for Mucopolysaccharide Diseases (the MPS Society), [The Niemann-Pick Disease Group \(UK\)](#) and the [Save Babies Through Screening Foundation UK](#). Our mission is to undertake joint promotion of LSDs; develop a shared understanding; advance standards of care; enhance the wellbeing of those affected; stimulate interest; and work in partnership to establish a Forum in which members can discuss together matters of common interest and contribute to the development and dissemination of good practice amongst them. Our key objective is to offer a strong voice with which to influence national policy.



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