Funded projects 2005

Strand 1

Strand 2

Strand 3

Project funded in 2005

European Myasthenia Gravis Network

Description - Financing - Outcomes - More info

Description

Action
Improving information and knowledge for the development of public health.

Area of activity
Developing strategies and mechanisms for preventing, exchanging information on and responding to non-communicable disease threats,including gender specific health threats and rare diseases.

Summary
Myasthenia Gravis (MG) is a rare and heterogeneous neuromuscular disease leading to abnormal fatigability of various muscles, with a prevalence of about 100 per million and an incidence which appears to be increasing. The cause of MG could be genetic or aquired. The genetic forms incriminate several types of mutations in the acetylcholine receptor or associated proteins. Acquired MG has an autoimmune etiology; it is due to auto-antibodies to acetylcholine receptor found in 85% of patients, or other muscle endplate proteins such as MuSK. MG could appear in young adults, essentially females, while the late onset form is distributed equally in males and females. Treatment of autoimmune MG disease is symptomatic, with the use of cholinesterase inhibitors, and empiric, with the use of immunosuppressors, broad anti-immune therapy such as plasma exchange and intravenous immunoglobulins, and thymectomy for selected patients. Clinical classification of MG differentiates between purely ocular and generalised forms, but also between late and early onset forms. To this date, it is not possible to know if these forms are different entities or whether they are a part of a continuum. There are no differential diagnostic tests, the clinical features and evolution are very similar, and currently the same empiric therapies are used for all the groups.
A network concerning acquired MG has been created several years ago and was supported by the AFM (French association against myopathies). This project gave the basis of a structured coordinated network on MG including several scientific investigators and clinicians. Because this network has minimal budget, its activities were limited to exchanges between the experts in this field and an annual meeting. However, the development of this network has established the necessary background for developing a sustainable coordination in the area of health information, collection of epidemio-logical data, exchange of data and information within and between Member States. EuroMyasthenia project will cover essentially acquired MG, although hereditary MG patients could be integrated in a further step.

Several activities will be developed according to the main objectives:

1. Health Information. Referral laboratories will be established and experts will provide professional assistance and information throughout the European Community. A leaflet will be prepared for patients about their disease. An officially endorsed Website and a forum will be provided. It will be open to all citizens but access to database will be restricted for professionals. An electronic newsletter will be sent to all members every two months.
2. European database. EuroMyasthenia will result in the creation of a European database that will collect data from a large part of Europe and thus permit epidemiological studies, taking into account genetic, immunological, histological, clinical and hormonal aspects. Health indicators recommended by the ECHI-2 network and relevant to MG will be also included. At least 5000 patients from the European countries participating to this network will be included during the implementation of this project.
3. Epidemiological data collection. Epidemiological studies will be performed to monitor occurrence of the different forms of MG for individual countries and to identify new markers associated with disease pathogenic mechanisms. In turn, a list of recommended indicators will be established for the surveillance of Myasthenia Gravis in the member states and accession countries.
4. Standardization and Quality Assessment. European Guidelines for diagnosis and clinical management of MG will be prepared. A quality control system for laboratory diagnosis of the different forms of MG will be established. By educational and training visits in the expert centers, scientific and medical personnel will improve healthcare quality. In addition, the promotion of information between the different research groups will contribute to improve the knowledge of the immunologic and genetic pathological mechanisms of MG.

The long-term objectives of EuroMyasthenia are to

1. define accurately subgroups of MG patients,
2. optimise the therapeutic strategies,
3. propose new therapeutic protocols according to the clinical subgroup and
4. improve the diagnosis of MG patients.

To reach these objectives, Euromyasthenia brings together 25 groups who have made substantial contributions to the understanding and the management of Myasthenia Gravis and 6 organisations of patients. It includes 10 groups of researchers and 15 groups of clinicians with complementary expertise in neurology, immunology,

genetics, pathology, biochemical, hormonal and clinical fields. The originality of this project is to combine

1. scientific experts who will improve the knowledge,
2. clinicians who will promote the databases and the epidemiological studies and
3. organisations of patients who will directly be informed on the progress of the project.

Finally such coordinated action on a European level will provide a solid evidence base for healthcare management, and will enhance the efficacy of healthcare systems all over Europe by exchanges with members from regulatory authorities from the represented countries.

More info...

Description - **Financing** - **Outcomes** - **More info**

Financing

Leader organisation
Université de Paris – Sud XI (UPS)
Public body
15 rue Georges Clémenceau
91405 ORSAY CEDEX
FRANCE
Tel: +33 1 69 15 74 06
Fax: +33 69 15 61 03
Web site: **www.u-psud.fr**

Contact Person
Dr Sonia BERRIH-AKNIN
Université Paris-Sud
CNRS UMR 8162
Centre Chirurgical Marie Lannelongue
133 avenue de la Résistance
92350 Le Plessis-Robinson
FRANCE
Tel: +33 1 45 37 15 51
Fax: +33 1 40 94 67 54
E-mail: **sonia.berrih@ccml.u-psud.fr**

Associated beneficiaries

- Bayerische Julius-Maximilians-Universität Würzburg (UW) - established in Würzburg, Germany
- Hellenic Pasteur Institute (HPI) - established in Athens, Greece
- Universitet I Bergen Sedd (UB) - established in Bergen, Norway
- Karolinska Universitetssjukhuset (KU) – established in Stockholm, Sweden
- Universiteit Maastricht (UM) – established in Maastricht, The Netherlands
- Ulleval Universitetssykehus (UU) – established in Oslo, Norway
- Istituto Nazionale Neurologico “Carlo Besta” (NNI) – established in Milan, Italy
- MDA-Hellas (MDA) – established in Athens, Greece
- The Chancellor, Masters and Scholars of the University of Oxford (The University of Oxford) - established in Oxford, United Kingdom

Starting date and duration of project
- 01/01/2006

- 36 months

Total cost
1.302.708,36 €
Subsidy from the Commission
770.400,00 €

Description - **Financing** - **Outcomes** - **More info**

Outcomes

Results to be achieved
Work package 1: Coordination of the project
This workpackage is linked to all objectives:

1. To promote the development of the Internet site and the database (objectives 3 and 5)
2. To ensure that deliverables and milestones are obtained in time and that the studies are performed in conformity with the National and European Regulations (all objectives)
3. To check the justification of the budget in relation to the technical tasks (all objectives)
4. To organize the network meetings (all objectives)
5. To set up an internal evaluation committee and an external evaluation and advisory board to evaluate the progress of the network (all objectives)

Work package 2: Dissemination of the results
This workpackage is linked to the objectives 1, 2, 3, 4 and 5:

1. To standardize biological, clinical and histological criteria all over the EU countries
2. To promote a survey among the associations of MG patients to determine the influence of psychological and socio-economical determinants in the onset or aggravation of the disease
3. To develop a database at a European scale including biological and clinical parameters
4. To promote the establishment of a specific European Card for MG patients
5. To disseminate data via a web-site connected to the EU health portal

Work package 3: Evaluation of the project
External Evaluation and Advisory Board will consist of eminent scientists, clinicians and association representatives. In addition, representatives from relevant clinical and scientific societies (e.g. International Society for Neuroimmunology, MGA, AIM) will be asked to participate. They will contribute by evaluating the progress of the network and will be consulted periodically for major directions of the project. Individuals we are considering to invite include: J. Newsom-Davis, H. Wekerle, D. Drachman, A. Lanzavecchia, J.F. Bach.

Questionnaires will be sent to 500 myasthenic patients from all participant countries. They will be asked about their current status and their expectations from the present Myasthenia network. Another questionnaire will be sent to 100 neurologists with special interest on MG. They will be asked for the difficulties and problems while working with myasthenic patients. After the first 18 months of the network and at the end of the project, new questionnaires will be prepared, asking the same people (patients or clinicians) to evaluate the results of our network. Their responses will be analyzed and evaluated by the external and internal Committees.

Work package 4: Database
The aim of WP4 is the realization of a European Database on MG (EuroMyasthenia-DB) that collects clinical and biological information to be shared among scientists and clinicians. The EuroMyasthenia-DB will allow the interaction of the European national registries and hospital databases containing country-specific epidemiological, clinical and biological data. EuroMyasthenia-DB will be a crucial tool for the establishment of a European cohort of MG patients to be used for a comprehensive understanding of the disease natural history, Public Health evaluation, cost-care effectiveness and treatment innovation.
This workpackage is linked to objective 3

Work package 5: Laboratory and clinical standardisation
This workpackage will be the driving force for the objective 1, that is to standardise biological, clinical and histological criteria all over the EU countries and will contribute data to objective 3 that is to develop a database on a European scale. The work done in this workpackage will be crucial for the improved diagnosis and clinical assessment of patients in those countries in which MG does not have strong research or laboratory links.

Work package 6: Psycho-socioeconomical determinants
To identify psychological and socio-economical determinants that could influence the development of MG disease and the clinical severity, a questionnaire will be established and will be sent to 3000 patients through the associations of patients.
This workpackage is linked to objectives 2 and 3.

Work package 7: Epidemiological analysis
This workpackage is linked to objective 3: develop a database at a European scale to serve as a basis of epidemiological studies

Interim report pdf (4 MB)

Description - **Financing** - **Outcomes** - **More info**

More info

Statement of project aim(s) and objectives
General objectives
Myasthenia gravis (MG) is a disabling chronic neuromuscular disease affecting mostly females. There is a need for combining the efforts to improve the knowledge on this disease for the following reasons:

1. MG is a rare disease (prevalence about 10/100 000);
2. it is heterogeneous;
3. it could be life-threatening, when the respiratory muscles are affected;
4. the diagnosis is not always easy to make;
5. the clinical criterion and therapeutic approaches are different among the European countries;
6. there are aspects in the pathogenesis of the disease that are not yet clear;
7. there is no cure for this disease;
8. many of the drugs used for the treatment of MG have severe side-effects
9. some pharmaceutical molecules, such as anaesthesia agents are life-threatening for the patients.

Our main objectives are:

1. to improve knowledge and information on the different forms of MG
2. to improve standardisation of data collection
3. to promote education and training, namely for the integration of new member states
4. to identify important health indicators for MG
5. to collect data and perform epidemiological studies at European level in order to improve the classification of MG disease' subtypes.

The strategy of EuroMyasthenia is to bring together 31 groups from 14 different countries, including 3 major categories:

1. scientists who have made substantial contributions to the understanding of MG;
2. clinicians experienced with the management of MG patients;
3. associations of MG patients.

The members of the network will join forces to construct a database including some indicators of health recommended by the ECHI-2 network as well as clinical, biological and psycho-socioeconomical factors collected by the members of the network. Further analyses of this database will enable to achieve main objectives of the EU call and promote a better classification and treatment of MG patients.
A better knowledge of the disease and more detailed information for the general practioners and neurologists should avoid erroneous diagnosis, and improve treatment and health. In the long term, such coordinated action on a European level will provide a solid evidence base for healthcare management, and will enhance the efficacy of healthcare systems all over Europe.

Specific objectives

Improving exchange of information and promoting a better classification is a priority for Europe (2.1.4 in Work Plan 2005). EuroMyasthenia aims to set-up a Europe-wide Myasthenia Gravis (MG) network to improve information and knowledge for MG, promote a better classification, optimize therapeutic strategies and support actions to reduce inequalities in MG care.

The specific objectives of EuroMyasthenia are to:

1. standardize biological, clinical and histological criteria all over the EU countries
2. promote surveys among the organisations of MG patients to determine the influence of psychological and socio-economical determinants in the onset and/or aggravation of the disease
3. develop a database at a European scale to serve as a basis for epidemiological studies
4. promote the establishment of a specific European Card for MG patients
5. disseminate data via a web-site connected to the EU health portal

These objectives will be achieved by

- Building on complementary experience of 10 scientists expert in the field of Myasthenia Gravis (Objectives 1, 3, 5), of 15 teams of clinicians (objectives 1, 2, 3, 4, 5) and of 6 organisations of patients (objectives 2, 4, 5).
- Reinforcing the exchange of information via an Internet Website, by exchanges of laboratory, medical and paramedical personnel, by the production of a regular newsletter and organisation of regular network meetings and an international conference.
- Supporting close collaboration with other networks supported by the EU on rare diseases (Orphanet and Eurordis).
- Establishing questionnaires to be sent to MG patients and neurologists among the associated partners and collaborators of the project, but also among new European countries that could be integrated to the project during the implementation.
- Producing a European set of Guidelines for Myasthenia Gravis diseases. This will be possible by the participation of members from regulatory authorities from the represented countries.

Methods
The creation of an internet site available to the European health portal will be based on previous European experience in Public Heath networks (WP1 and WP2). A part of this site will be open to all citizens. It will include all information reporting the progress of the project and a forum.

The psycho-socio-economical determinants will be analysed through a questionnaire prepared in several languages and sent to MG patients through the organisations of patients (WP6). Finally the epidemiological analysis will be implemented with the help of technical competences of several collaborators (WP7).

The European database will be based on national databases, and will include Standardized clinical, biological and genetic data from MG patients (WP4). Indicators recommended by the ECHI-2 network will be introduced. Collaborating with other European projects, in particular Orphanet and Eurordis will promote synergy and avoid duplication.

To standardise the clinical and biological parameters, reference reagents will be distributed to the centers performing the tests and personnel will be educated in expert centers (WP5). Methodological problems will be discussed during the meetings.
Evaluating the progress of the project will be done through the external Evaluation and Advisory Board (WP3). The preparation of the various Guidelines and leaflets will be discussed during the meetings. All reports and communication documents will be under the supervision of the internal evaluation committee.

This project brings together multiple competences and will enrol several personnel to fit the tasks described in the workpackages. However for specific technical tasks such as the creation of a professional website or database, the internal evaluation committee could decide whether subcontracting with SMEs is required.