The Rare disease patient solidarity project

New services aimed at improving the daily lives of people living with a rare disease

A project by Eurordis and its 9 partners

www.eurordis.org
The overall objectives of Rapsody are to improve the quality of care, information and social services for people living with a rare disease.

- offering quality information provided by rare disease help lines throughout Europe and helping very isolated persons make contact with others suffering from a similar disease
- standardising the quality of therapeutic recreation programmes for children and young adults living with a rare disease.
- guiding school professionals on how to accommodate children living with a rare disease within the school curriculum
- identifying respite care services as well as creating networks for severely disabling rare diseases
- measuring patients' expectations on the administration of medical, paramedical and social care, through a pan European survey, tailored to the needs of thirteen specific rare disease networks
- participating in the European reflection process on national centres of reference and European networks of reference for rare diseases

Rapsody is the result of this long term commitment with the creation of databases with user friendly internet interfaces, cartographic displays of the services developed by participants, and health policy proposals to European institutions and member states.

The Rare Disease Patient Solidarity Project is committed to:

The Baxter International Foundation
Sigma Tau Pharmaceuticals
Actelion
Union Groupe Initiatives Mutuelles

Partners / credits
European Commission, DG Health and Consumer Protection
Eurordis
Fundacion Doctor Robert UAB, Spain
Children Living with Inherited Metabolic Diseases Climh, United Kingdom
Federacion Espanola De Enfermedades Raras, FEDER, Spain

Sponsors
The Baxter International Foundation
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Barretstown, Ireland
Frambu, Norway
Rare Disorders Denmark, Denmark
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Association Francaise contre les Myopathies, AFM, France
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Help Lines for Rare Diseases

In the absence of widely available information on their respective disease it is vital that services exist to respond to the needs of rare disease patients, an often ignored section of society, whether it be someone who needs specific medical information about their disease or simply someone who is struggling to cope and needs to hear a voice on the other end of the line.

Rapsody will bring together representatives from Helpline services from various European member states with the aim of creating a common databases with regularly updated statistics on the scope, approach, location, organisation, etc. of each service.

The project will look at national models designed to put very isolated patients in touch with each other and create a similar service on a European level.

This part of the project will also attempt to standardise an evaluation procedure to assess whether respondents are offering the best possible service to their callers through the use of a self-evaluation questionnaire.
Barretstown in Co. Kildare, Ireland is a specially designed camp, providing a programme of adventure activities and fun which helps children with serious illness regain their confidence and self-esteem.

There are camps offering a similar service to Barretstown all over Europe. The Rare Disease Patient Solidarity Project will bring representatives of these programmes together to create a European network with the goal of:

- Increasing awareness of therapeutic recreation programmes, for example, as a treatment option within the medical community
- Learning from each other and creating ‘best practice’ and standards
- Creating an online service with a cartographic representation of the various services and their location.
- Facilitate the movement of patients across European borders to an ever increasing range of different types of programmes.

A core group of programme organisers will identify the organisations that offer services which fit into a predefined set of criteria of what a therapeutic recreation programme means and then add them to the online database. This service is also supported by the Baxter International Foundation.

The Rare Patient Solidarity Project will create a network of existing respite services (education, learning and habilitation) which provide for patients and families living with a rare disease. Respite care includes, for example, nursing care delivered to inpatients who need assistance with activities of daily living on a continuing basis due to chronic impairment and a reduced degree of independence.

Care can be provided in institutions or community facilities with a mix of medical and social services. Rapsody facilitates a dialogue in which the identified centres wishing to collaborate will have the opportunity to share their needs, concerns and challenges and discuss possibilities of joint learning and development of projects at EU level.

Many families do not have access to appropriate services. An investigation process will be conducted in order to identify the obstacles which make it difficult for the centres to expand the scope of services offered to RD patients who do not currently have access to the services. This process will conclude with the development of a set of best practices to assist in overcoming the obstacles.
Supervised by François Faurisson, research engineer at Inserm, a large patient survey is being launched to measure expectations concerning access to care, access to specialised care centres, and the patients’ satisfaction (EurordisCare survey programme).

A questionnaire distributed by 98 patient groups belonging to the following diseases:

- Marfan syndrome
- Fragile X syndrome
- Williams syndrome
- Ehlers-Danlos syndrome
- Cystic fibrosis
- Prader-Willi syndrome
- Epidermolysis bullosa
- Tuberous sclerosis
- Osteogenesis imperfecta
- Huntington disease
- Proximal spinal muscular atrophy
- Friedreich ataxia
- Aniridia
- Pulmonary arterial hypertension
- 11q deletion syndrome
- Alternating hemiplegia

This survey is conducted with the technical support of an advisory committee led by Rosa Sanchez de Vega. It is also supported by Actelion Pharmaceuticals and Union Groupe Initiatives Mutuelles.

The High Level Group on Health Services and Medical Centres set up by the European Commission and member states has come up with some principles regarding European centres of reference, including their role in tackling rare diseases or other conditions requiring specialised care and volumes of patients and some criteria that such centres should fulfil. Options and procedures for designating European centres of reference for limited periods of time at European level based on agreed lists of pathologies, technologies and techniques are also being developed. The High Level Group will work towards a common approach which could then be implemented through pilot activities.

Its reflection is based on a report from the working group of the Rare Diseases Task Force first published in September 2005 and recently updated (September 2006).

Rapsody is organising the dialogue both at national and European level:
Ten meetings are organised in ten different member states, with the same agenda, same format, and composed of the same audience: 20 representatives of patients, health care professionals, health care systems experts and health authorities.

After each meeting, a synthesis will be prepared and presented at a European workshop in Prague, July 12th–13th 2007. This two-day workshop will prepare the debates of the European Conference on Rare Diseases in Lisbon in November 2007, and a report to European policy makers.
Ensuring that Rapsody meets the needs of the rare disease community

Experts from the London School of Economics (LSE), Panos Kanavos and Ömer Saka are conducting the project evaluation, together with a panel of patients. The evaluation of the project, of its impact, of its completion, is key to improve future efforts. As it is partly funded by public funding, the beneficiaries and the European Commission are committed to demonstrate its utility. Therefore, we have asked for an independent evaluation by an institution that is not related to Eurordis.

The questions to answer are:

→ Has the project met its stated objectives?
→ Have the new tools been efficient in delivering their objectives?
→ Are the new tools useful for the patients, families and patient organisations?
→ Has the project served the needs of rare disease patients?
→ What is the European added-value of the project?

Responses will be part of the final report to the European Commission and partners.

Contact

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About EURORDIS

The European Organisation for Rare Diseases (EURORDIS) represents more than 260 rare disease organisations in over 30 different countries, covering more than 1,000 rare diseases. It is therefore the voice of the 30 million patients affected by rare diseases throughout Europe.

EURORDIS is a non-governmental patient-driven alliance of patient organisations and individuals active in the field of rare diseases, dedicated to improving the quality of life of all people living with rare diseases in Europe. It is supported by its members and by the French Muscular Dystrophy Association (AFM), the European Commission, and corporate foundations and the health industry. EURORDIS was founded in 1997. Further details concerning EURORDIS and rare diseases are available at:

http://www.eurordis.org

Rapsody activities are supervised by a steering committee:

Yann Le Cam, Eurordis
François Houÿez, Eurordis
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Prof Josep Torrent I Farnell, Fundacio Dr Robert, Spain
All aspects of Rapsody and much more will be addressed during the European Conference on Rare Diseases in Lisbon on the 27 & 28 November 2007.

Themes:

- Living with a Rare Disease, patients’ expectations of health services
- Therapeutic Recreation Programmes
- Respite Services
- European Policy for Rare Diseases, Centres of Reference
- Research
- Orphan drugs
- Information and help lines

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More information on projects supported by the Public Health Programme of the European Communities, DG Sanco:


More information on health in Europe, the European Public Health Portal:

http://ec.europa.eu/health-eu/index_en.htm

For printed versions, please contact Eurordis.

www.eurordis.org
**European Conference**  
**on Rare Diseases Lisbon 2007**  
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Prof Josep Torrent i Farnell, Fundacio Dr Robert

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Katrine Fyhri, Frambu

**Therapeutic Recreation Programmes:**  
Terry Dignan, Barretstown

**Help Lines for Rare Diseases:**  
Pamela Davies, Climb

**Patients' Survey EurordisCare:**  
Rosa Sanchez deVega, FEDER

**Centres of Reference:**  
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**EU Public Health Programme**

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**And also:**

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SUKL, Jitka Sabartova  
AFM-Téléthon, Tuy Nga Brignol