



**Social Economic Burden and Health-related
Quality of Life in Patients with
Rare Diseases in Europe
(BURQOL-RD)**

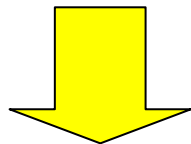
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October, 2011



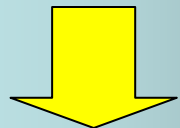
Background

“Council Recommendation on a European action in the field of rare diseases”
2009/C 151/02



EUROPLAN (2008 – 2011)
To develop recommendations to elaborate national plans for RD, to facilitate the coherence of national initiatives with best practices.

Need of **tools to measure the impact** of the national policies based on the recommendations of EUROPLAN



BURQOL RD
(2010 – 2013)

Main Aim: Model

BURQOL-RD aims to generate a model to quantify the socio-economic burden and HRQOL of **both RD patients and caregivers.**

- The model will be generated in **10 RD in 8 different European countries.**
- This model will be **adaptable and sufficiently sensitive** to capture the differences in the distinct Health and Social Care Systems in the EU Member States.



BURQOL-RD Project

BURQOL-RD is a 3 year project under the 2nd Programme of Community Action in the Field of Public Health, that commenced in April 2010.

- Funded by the **Executive Agency for Health and Consumers** (EAHC)
- **Project coordinator:** Prof. Julio López Bastida
- **Coordinator:** Fundación Canaria de Investigación y Salud (FUNCIS), Canary Islands, Spain.



Associated Partners



- Instituto Superior di Sanita (Italy)
- London School of Economics and Political Science (UK)
- Leibniz University Hannover (Germany)
- Federación Española de Enfermedades Raras (Spain)
- The Swedish Institute for Health Economics (Sweden)
- University Paris val de marne (France)
- Centre for Public Affairs Studies Foundation (Hungary)
- Instituto de Salud Carlos III (Spain)
- Università Commerciale "Luigi Bocconi" (Italy)
- Mario Negri Institute for Pharmacological Research (Italy)
- Bulgarian Association for Promotion of Education and Science (Bulgaria)

Associated Partners are responsible for carrying out all the core activities of the project.

Collaborating Partners



- National Alliance of people with rare diseases (NAPRD, Bulgaria)
- Consulta Nazionale delle Malattie Rare (Italy)
- Federazione Italiana Malattie Rare (UNIAMO, Italy)
- Allianz Chronischer Seltener Erkrankungen (ACHSE, Germany)
- Rare Diseases Sweden (SÄLLSYNTA DIAGNOSER, Sweden)
- Hungarian Federation of People with Rare and Congenital Diseases-Rare Diseases Hungary (HUFERDIS, Hungary)
- Rare Diseases UK-Genetic Interest Group (GIC, United Kingdom)
- Alliance Maladie Rares (France)
- Euro-Histio-Net
- CRE Enfermedades Raras (Spain)

We are especially grateful to EURORDIS for their collaboration and assistance that they provide in certain areas fundamental for the development of the project.

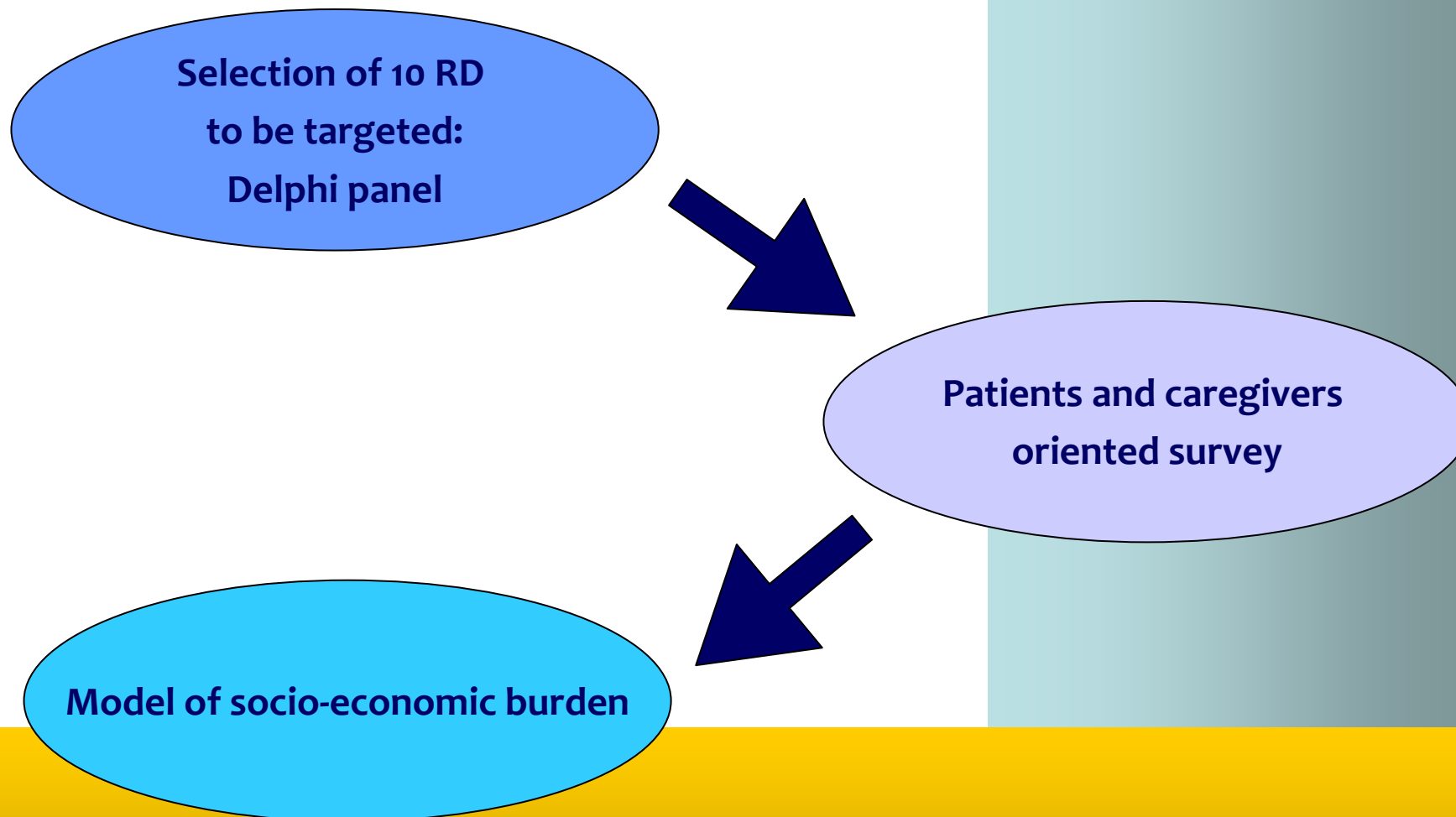
Collaborating Partners contribute to specific activities in the project and provide advice and assistance to ensure that all the objectives proposed are successfully reached.

Specific objectives

- I. To generate a framework **to measure the socio-economic burden and the HRQOL** of RD.
- II. To develop **unified instruments** to gather information on the socio-economic burden and HRQOL of RD throughout Europe.
- III. To perform **a pilot study measuring the socio-economic burden and HRQOL** for selected RD.
- IV. To refine and package the tools **for the continued study of the costs and HRQOL of RD.**

The collaboration of National Patient Associations and Federations for the specific RD is fundamental to ensure that all the objectives proposed are successfully reached.

Development



Target RD

Combining the Delphi prioritization of RD and the Carroll diagram →

Final set of RD to be targeted in the study:

- Cystic fibrosis
- Prader-Willi Syndrome
- Haemophilia
- Duchenne Muscular Dystrophy
- Epidermolysis Bullosa
- Fragile X Syndrome
- Scleroderma
- Mucopolysaccharidosis
- Juvenile idiopathic arthritis
- Histiocytosis

Survey: Patients

- Patients and carers oriented survey
- Patients:
 - Burden of disease (socioeconomic costs)
 - E.g. drugs, medical tests, transport, hospitalization, etc.
 - Health related quality of life
 - EQ-5D for adults
 - EQ-5D-Y for children between 6 and 17
 - Disability
 - Barthel Index
 - Demographic data

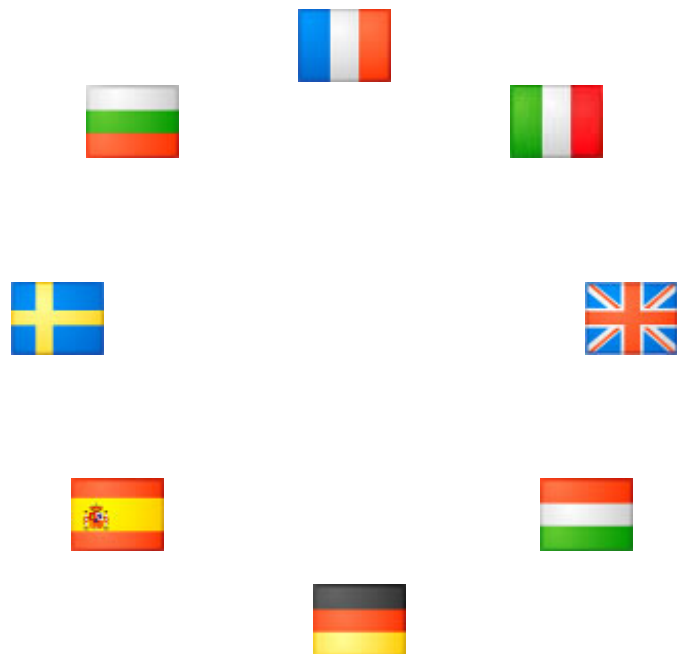


Survey: Caregivers

- Caregivers
 - Health related quality of life
 - EQ-5D
 - Over-burden of the caregiver
 - Zarit Scale
 - Time utilization
 - Demographic data



On-line survey in Europe



- Launched in July in Spain
- Expected to be launched in Autumn 2011 in:
 - France
 - UK
 - Italy
 - Sweden
 - Germany
 - Hungary
 - Bulgaria

The **collaboration of national patients' association and federations** is fundamental to ensure that all the objectives are successfully reached.

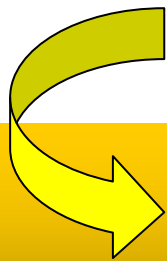
Expected outcomes

1. An integrated and harmonized **set of instruments to assess and monitor socio-economic burden and HRQOL** of patients affected by RD and their caregivers.
2. A detailed **analysis of the services** (health and social care) received by people with specific RD in different EU countries, including the identification of formal and informal care.
3. A report on the **current socioeconomic and HRQOL status of RD patients and caregivers** for the selected RD and EU countries.

The results and deliverables that emerge from this project will stimulate the **future comparability and monitoring of RD** in Europe as well as anticipate future information needs.

Main benefits


- The BURQOL-RD model will provide an **integrated and harmonised** means to assess the impact of new **public health policies**, interventions and treatments for RD “in” and “among” EU Member States.
- Moreover, the associated dissemination activities undertaken by BURQOL-RD will also **improve RD awareness and literacy** among European citizens.



VALIDATED TOOLS

MORE VISIBILITY


www.burqol-rd.com




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 **SOCIAL ECONOMIC BURDEN AND HEALTH-RELATED QUALITY OF LIFE IN PATIENTS WITH RARE DISEASES IN EUROPE**

BURQOL-RD is a 3 year project under the 2nd Programme of Community Action in the Field of Public Health, that commenced in April 2010 and is promoted by the DG Sanco.

The main aim of BURQOL-RD is to generate a model to quantify the socio-economic costs and Health Related Quality of Life (HRQOL) , of both patients and caregivers, for up to 10 rare diseases in different European countries.

This model will be adaptable and sufficiently sensitive to capture the differences in the distinct Health and Social Care Systems in the EU Member States. Our hope is that, the information generated by the BURQOL-RD consortium will help to:

- Design future policies in the area of rare diseases, which will ultimately have positive benefits for EU citizens health, both that of patients and of their caregivers.
Readily transfer the protocols established to other RD and to other countries.
Compare the availability and access to specific health resources for specific RD in each country.
- Explore the potential relationships between HRQL and access to healthcare resources.

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