

BURQOL-RD

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



Fundación Canaria de Investigación
y Salud (FUNCIS), Spain.

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.

- **Project coordinator:** Julio López Bastida (Senior Researcher in Health Technology Assessment Unit, Canary Islands Health Service).
- The Coordinator will be supported by the **Steering** and the **Advisory Committees**.

We are especially grateful to **Eurordis** for their Collaboration and the assistance that they will provide during the development of the project.

Background

- Rare Diseases (RD) have a low prevalence - $\leq 5/10,000$ persons.
- There are more than 5000 RD and hence, over 30 million EU citizens suffer from these conditions.
- Most RD are life-threatening, chronic and debilitating, requiring long term, specialist, and costly formal and informal care.
- The lack of effective diagnoses and treatments often underlies the shorter life expectancy and quality of life of these patients.
- Together, these characteristics mean that RD require the combined efforts of health and social care professionals, politicians and researchers to improve care and to extend both life expectancy and Health Related Quality of Life (HRQOL).

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 up to **10 RD 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.
- The tools developed by BURQOL-RD will build on the ongoing EUROPLAN project and BURQOL-RD will also incorporate strategies previously developed by EURORDIS.

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.

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 Association's and Federations for the specific RD's to be addressed in the project is fundamental to ensure that all the objectives proposed are successfully reached.**

Target groups

The target groups that will benefit from the activities carried out in BURQOL-RD will include:

- RD Patients
- RD Patient Caregivers
- RD Patient Associations
- National and EU Health Authorities
- Experts within the EU from the field of
 - *Health economics*
 - *Health related quality of life*
 - *Health planning and epidemiology*

These target groups will also provide input into the project in terms of:

- 1) RD selection and prioritization;
- 2) Integration and adaptation;
- 3) Patient Recruitment.

Given the nature and the goals of the project, it is clear that a **fundamental beneficiary of the results** of this project will be the **families and caregivers of those affected by RD**, a group that is often overlooked when considering such devastating diseases.

Methods and Means - I

Data on **Socioeconomic burden** will be collected as in previous studies (Eurordiscare I-III).

This will include:

- Direct costs - **medical resources** (e.g. hospitalisation, consultations, drugs).
- Direct costs - **non-medical resources** (e.g. walking sticks, wheelchairs, modifications to house and car, home care, transport, care by relatives).
- Indirect costs - including **morbidity** (productivity losses) and **mortality costs**.
- Intangible costs - measured by comparing the patients' HRQOL to those of an age and gender matched sample of the general population.

Methods and Means II

HRQOL data will be collected from patients registered in RD associations using a **standardized pre-tested questionnaire** and/or a widely used generic preference-based tool (EQ-5D)* addressing 5 **HRQOL domains**:

- **Mobility**
- **Self-care**
- **Usual activities**
- **Pain/discomfort**
- **Anxiety/depression**

The overall HRQOL will be measured both in RD patients and their caregivers.

*Reference scores for the EQ-5D are available for a number of European countries.

Expected Outcomes

- 1.- The production of **integrated and harmonized instruments** to assess and monitor socio-economic burden and HRQOL associated with RD, both in patients and their caregivers.

These instruments will help measure the impact of new policies, interventions, treatments and diagnostic techniques for different RDs and EU member states, permitting extrapolation to many RD.

- 2.- A detailed analysis of the **health and social care services 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.

Strategic relevance + Added value

The information generated by the BURQOL-RD consortium will be critical to:

- **Design future policies in this area**, 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.**

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)

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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)

