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. These instruments will help measure the impact of new policies, interventions, treatments and diagnostic techniques, adapted to different RD and EU member states/languages, permitting the extrapolation to the rest of the EU and to a wide range of RD.
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.

The outcomes will be made readily accessible and posted on the websites of the participating Patient Associations, BURQOL-RD, EUROPLAN and EC public web-sites. The outcomes will also be distributed to health policy makers in the EU, while the scientific outcomes will be disseminated by means of scientific papers and meetings.

www.burqol-rd.com

BURQOL RD

More information: **info@burqol-rd.com**

BURQOL RD

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

www.**burqol-rd**.com

Aims

The main aim of BURQOL-RD is **to generate a model to quantify the socio-economic costs and HRQOL**, of both patients and caregivers, for ten RD in eight 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. As such 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.

The tools developed by BURQOL-RD will build on the ongoing EUROPLAN project and BURQOL-RD will also incorporate strategies previously developed by EURORDIS. Moreover, the associated dissemination activities undertaken by BURQOL-RD will also improve RD awareness and literacy among European citizens.

Given the nature and the goals of the project, it is clear that the 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.

The **collaboration of national patients' associations and federations** for the specific RDs to be addressed in the project is fundamental to ensure that all the objectives are successfully reached.

Target RD

The RD targeted in the pilot study of BURQOL-RD are:

- **Cystic Fibrosis**
- **Prader-Willi Syndrome**
- **Haemophilia**
- **Scleroderma**
- **Epidermolysis Bullosa**
- **Histiocytosis**
- **Juvenile Idiopathic Arthritis**
- **Mucopolysaccharidosis**
- **Fragile X Syndrome**
- **Duchenne Muscular Dystrophy**

A **survey on patients and caregivers** will be held in these countries:

- **Spain**
- **France**
- **UK**
- **Italy**
- **Sweden**
- **Germany**
- **Hungary**
- **Bulgaria**

BURQOL-RD

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Despite the low prevalence of Rare Diseases (RD) (5/10,000 persons), more than 5000 RD are recognised and hence, over 30 million EU citizens suffer from these conditions.

Most cases 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 shortened life expectancy and quality of life of these patients.

These characteristics mean that RD require the combined efforts of health and social care professionals, politicians, managers and researchers to increase the availability of effective disease management tools to improve care and to extend both life expectancy and Health Related Quality of Life (HRQOL).

Main partner

Canary Foundation of Investigation and Health (FUNCIS), Spain.

The Coordinator is supported by the **Steering Committee (SC) and the Advisory Committee (AC)**.

Associated partners

- Instituto de Salud Carlos III (ISCIII) – Research Institute for Rare Diseases (IIER), Spain
- Instituto Superiore di Sanita (ISS), Italy
- London School of Economics and Political Science (LSE-Health), UK
- Bulgarian Association for Promotion of Education and Science (BAPES), Bulgaria
- Federación Española de Enfermedades Raras (FEDER), Spain
- Leibniz University Hannover (LUH). Germany
- The Swedish Institute for Health Economics (IHE), Sweden
- Universita Commerciale "Luigi Bocconi" (Bocconi), Italy
- University Paris Val de Marne (UPVM), France
- Centre for Public Affairs Studies Foundation (CPASF), Hungary
- Mario Negri Institute for Pharmacological Research (IRFMN), Italy

Collaborating partners

- National Alliance of people with rare diseases (NAPRD), Bulgaria
- Consulta Nazionale delle Malattie Rare, Italy
- UNIAMO (Federazione Italiana Malattie Rare), 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), UK
- CRE Enfermedades Raras (CREER), Spain
- Euro-Histio-Net, a international reference network for Langerhans cell histiocytosis and associated syndromes
- European Organisation for Rare Diseases (EURORDIS)

Funds

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