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# European Organisation for Rare Diseases

JA2015 - GPSD [705038]

START DATE: 01/01/2011

END DATE: 01/01/2012

DURATION: 12 month(s)

CURRENT STATUS: Finalised

PROGRAMME TITLE: Second Programme of Community action in the Field of Health 2008-2013

PROGRAMME PRIORITY: -

CALL: Promote Health (Hp-2010)

TOPIC: PROMOTE HEALTH (HP-2010)

EC CONTRIBUTION: 580000 EUR

KEYWORDS: Accessibility, Health system, Patients rights, Pharmaceuticals, Rare diseases and disorders

PORTFOLIO: Rare diseases

## Mission and vision of the operating grant holder

EURORDIS is a patient-driven alliance of Patient Organisations (POs) and individuals active in the field of rare diseases (RD). It represents the voice of an estimated 29 million citizens in the EU.

EURORDIS mission is to build a strong pan-European community of Patient Organisations and people living with RD, to be their voice at the European level and to fight against the impact of RD on their lives.

To this end, EURORDIS undertakes activities on behalf of its members:

- Empowering RD patient groups
- Advocating RD as a public health priority
- Raising public awareness on RD
- Improving access to information, treatment, care and support for people living with RD
- Improving quality of life
- Encouraging good practices in relation to these issues
- Promoting scientific and clinical research on RD
- Developing treatments and drugs for people with RD

EURORDIS plays a pivotal role in the implementation of the EU strategy on RD (EC Communication, Dec.2008 and Council Recommendations, June 2009), in the EU Committee of Experts on RD and in the elaboration of National Plans or Strategies on RD.

## Strategic objectives and specific activities

### 1. Consolidate the EU RD patients community & voice

Promote RD as a public health priority at national European & global levels  
 Be the voice of all RD patients, genetic or not, incl. ultra RDs and rare cancers  
 Raise public awareness on RD, incl. Rare Disease Day  
 Organise the ECRD every 2 years  
 Focus on key EU policy issues relevant to people living with RD

### 2. Facilitate the implementation of EU regulations/strategies

Support effective implementation of EC Communication & Council Recos on RD  
 Promote RD national plans  
 Evaluate impact of regulations & strategies on RD patients  
 Participate in EU CERD & related WGs  
 Support therapeutic development/access to OD, paediatrics & ATMPs  
 Participate in EMA committees & WPs, bring in patient expertise  
 Inform & involve more members/volunteers in therapeutic activities  
 Facilitate dialogue between patient groups, experts, companies regulators, NCA & HTA  
 Support the development of EU/national integrative healthcare policies

Promote EU Ref Networks of Centres of Expertise and patient registries  
Participate in activities on screening & gene testing  
Support a better research policy framework  
Promote the development of EU research networks and infrastructures  
Support the participation of patients in research&ethics committee  
Support development&access to information & social specialised services  
Consolidate national Help Lines & EU Networking  
Produce validated information on respite centres & therapeutic recreation programmes  
Generate/share patient-based knowledge

3. Build capacities & empower members and volunteers  
Maintain high level of legitimacy and professionalism within the membership  
Organise the annual Membership Meeting  
Broaden the patient group membership base & focus on Central&Eastern Europe  
Identify, involve and support volunteers in more activities  
Intensify networking & collaborative process with/between National alliances and RD specific Federations  
Share good practices & organise training for patient reps

4. Sustain human, financial & organisational resources  
Good governance & financial transparency  
Web communications in work process  
Integrative IT infrastructures  
Diversify resources and increase public funding  
Increase in-kind resources

## Expected outcomes

A. Communication to POs, stakeholders and the public:

- a) EURORDIS Website & Electronic newsletter; 6 languages
- b) Preparation of the ECRD 2012 Brussels: Programme Committee, announcement, selection of Conference venue, hotels and providers
- c) Update of fact sheets
- d) RDD 2011 on 28/02 and Media Monitoring Services; Rare Disease Day 2012 preparation
- e) Awareness raising through patient stories, video & photo
- f) Maintenance of EURORDIS RD community databases: 1400 POs, 406 members, 150 volunteers
- g) International dialogue with NORD, CORD, ICORD, DIA, etc.
- h) Promoting RD as an international priority

B. Capacity building for patient representatives:

1. Health Policy, POs networking and empowerment:

- a) Outreach to POs and members, dissemination of information, consultation of

members

- b) Strengthen focus on new MS, Eastern & Central Europe; fellowships
- c) Support the EU Network of National Alliances; organisation of 2 Workshops of the Council of National Alliances(CNA); involvement in public awareness (RD Day) and empowerment on the development of strategies for RD at national level
- d) Support the EU Network of RD specific Federations through dissemination and sharing of good practices; involvement in public awareness and empowerment on EU policies(CoE,ERN); organisation of 2 Workshops for the Council of European Federations(CEF)
- e) Support to volunteers representing EURORDIS in EU committees
- f) Fact sheets for capacity-building purposes on RD policy aspects

## 2. Research and Therapeutics Development:

### a) Patient involvement in EMA activities:

- Support the participation of patient representatives in the EMA Committees (COMP, PDCO, CAT) and Working Parties (Patients & Consumers, Protocol Assistance and Pharmacovigilance)
- Support the EURORDIS Therapeutic Action Group(TAG) composed of all RD patient representatives at the EMA to exchange information and coordinate actions
- Monthly report compiling feedback from each committee and WP

### b) Review and validate public information on RD therapies disseminated by the EMA at the time of designation (PSOs) and marketing authorisation (EPARs, Package Leaflet)

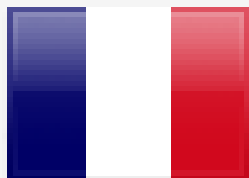
### c) Support involvement of more RD patient representatives in the OD,PD, ATMP policies and in drug development through information dissemination and capacity building activities on clinical trials and EU regulatory affairs, using the following means:

- Support EURORDIS Task Forces on Orphan Drugs, Pediatrics, Drug Information, Transparency & Access (DITA), each involving 10-15 trained volunteers
- Summer School 2011, based on experience exchange & case studies; 40 new participants
- e-Learning on specific and advanced aspects of drug development, clinical trials and regulatory affairs

### d) Support capacity building activities of patient advocates in HTA and access to medicines, through a section on the website, dissemination of information

### e) Support good practice relations between POs & Sponsors on RD Clinical Trials (CT) based on EURORDIS Charter on Clinical Trials (CCT): promote signature by pharmaceutical Cies, and provide adequate staff and advisors to support the collaboration on specific CTs

## COORDINATOR



European Organisation for Rare Diseases (European Organisation for Rare Diseases)

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## PARTNERS

No partners related to the current project

## D04 - Electronic Newsletter (EN)

European Organisation for Rare Diseases

European Organisation for Rare Diseases (EURORDIS\_FY2011)

Published on: 01/01/2011

[http://persond.cc.cec.eu.int:8082/publications/20103205/20103205\\_d04\\_oth\\_en\\_ps.pdf](http://persond.cc.cec.eu.int:8082/publications/20103205/20103205_d04_oth_en_ps.pdf)

## D01 - Activity Report EURORDIS FY2011 (EN)

European Organisation for Rare Diseases

European Organisation for Rare Diseases (EURORDIS\_FY2011)

Published on: 01/01/2011

[http://persond.cc.cec.eu.int:8082/publications/20103205/20103205\\_d01\\_oth\\_en\\_ps.pdf](http://persond.cc.cec.eu.int:8082/publications/20103205/20103205_d01_oth_en_ps.pdf)

## D02 - Workshops of the EU Networks (EN)

European Organisation for Rare Diseases

European Organisation for Rare Diseases (EURORDIS\_FY2011)

Published on: 01/01/2011

[http://persond.cc.cec.eu.int:8082/publications/20103205/20103205\\_d02\\_oth\\_en\\_ps.pdf](http://persond.cc.cec.eu.int:8082/publications/20103205/20103205_d02_oth_en_ps.pdf)

## D03 - Update of EURORDIS RD community Database (EN)

European Organisation for Rare Diseases

European Organisation for Rare Diseases (EURORDIS\_FY2011)

Published on: 01/01/2011

[http://persond.cc.cec.eu.int:8082/publications/20103205/20103205\\_d03\\_oth\\_en\\_ps.pdf](http://persond.cc.cec.eu.int:8082/publications/20103205/20103205_d03_oth_en_ps.pdf)

## D05 - EURORDIS Website (EN)

European Organisation for Rare Diseases

European Organisation for Rare Diseases (EURORDIS\_FY2011)

Published on: 01/01/2011

[http://persond.cc.cec.eu.int:8082/publications/20103205/20103205\\_d05\\_oth\\_en\\_ps.pdf](http://persond.cc.cec.eu.int:8082/publications/20103205/20103205_d05_oth_en_ps.pdf)

[\\_en\\_ps.pdf](#)

## D06 - Summer School 2011 and online training tools (EN)

European Organisation for Rare Diseases

European Organisation for Rare Diseases (EURORDIS\_FY2011)

Published on: 01/01/2011

[http://persond.cc.cec.eu.int:8082/publications/20103205/20103205\\_d06\\_oth\\_en\\_ps.pdf](http://persond.cc.cec.eu.int:8082/publications/20103205/20103205_d06_oth_en_ps.pdf)

## D07 - Meetings of the Task Forces Orphan Drugs, DITA, TAG, Paediatrics (EN)

European Organisation for Rare Diseases

European Organisation for Rare Diseases (EURORDIS\_FY2011)

Published on: 01/01/2011

[http://persond.cc.cec.eu.int:8082/publications/20103205/20103205\\_d07\\_oth\\_en\\_ps.pdf](http://persond.cc.cec.eu.int:8082/publications/20103205/20103205_d07_oth_en_ps.pdf)

## D08 - Participation in EMA Committees (COMP, PDCO, CAT, PCWP, PhVWP)(EN)

European Organisation for Rare Diseases

European Organisation for Rare Diseases (EURORDIS\_FY2011)

Published on: 01/01/2011

[http://persond.cc.cec.eu.int:8082/publications/20103205/20103205\\_d08\\_oth\\_en\\_ps.pdf](http://persond.cc.cec.eu.int:8082/publications/20103205/20103205_d08_oth_en_ps.pdf)

## D09 - Rare Disease Day 2011 (EN)

European Organisation for Rare Diseases

European Organisation for Rare Diseases (EURORDIS\_FY2011)

Published on: 01/01/2011

[http://persond.cc.cec.eu.int:8082/publications/20103205/20103205\\_d09\\_oth\\_en\\_ps.pdf](http://persond.cc.cec.eu.int:8082/publications/20103205/20103205_d09_oth_en_ps.pdf)

## Final Report (EN)

European Organisation for Rare Diseases

European Organisation for Rare Diseases (EURORDIS\_FY2011)



Published on: 01/01/2011

[http://persond.cc.cec.eu.int:8082/publications/20103205/20103205\\_d01\\_oth\\_en\\_ps1.pdf](http://persond.cc.cec.eu.int:8082/publications/20103205/20103205_d01_oth_en_ps1.pdf)