EURORDIS
Rare Diseases Europe

EU Major Policies from the patients' perspective & International engagements

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EURORDIS

The Voice of Rare Disease Patients

in EUROPE

Who are we?

An international non-profit, non-governmental umbrella rare disease patients' organisation representing an estimated 30 million individuals in Europe

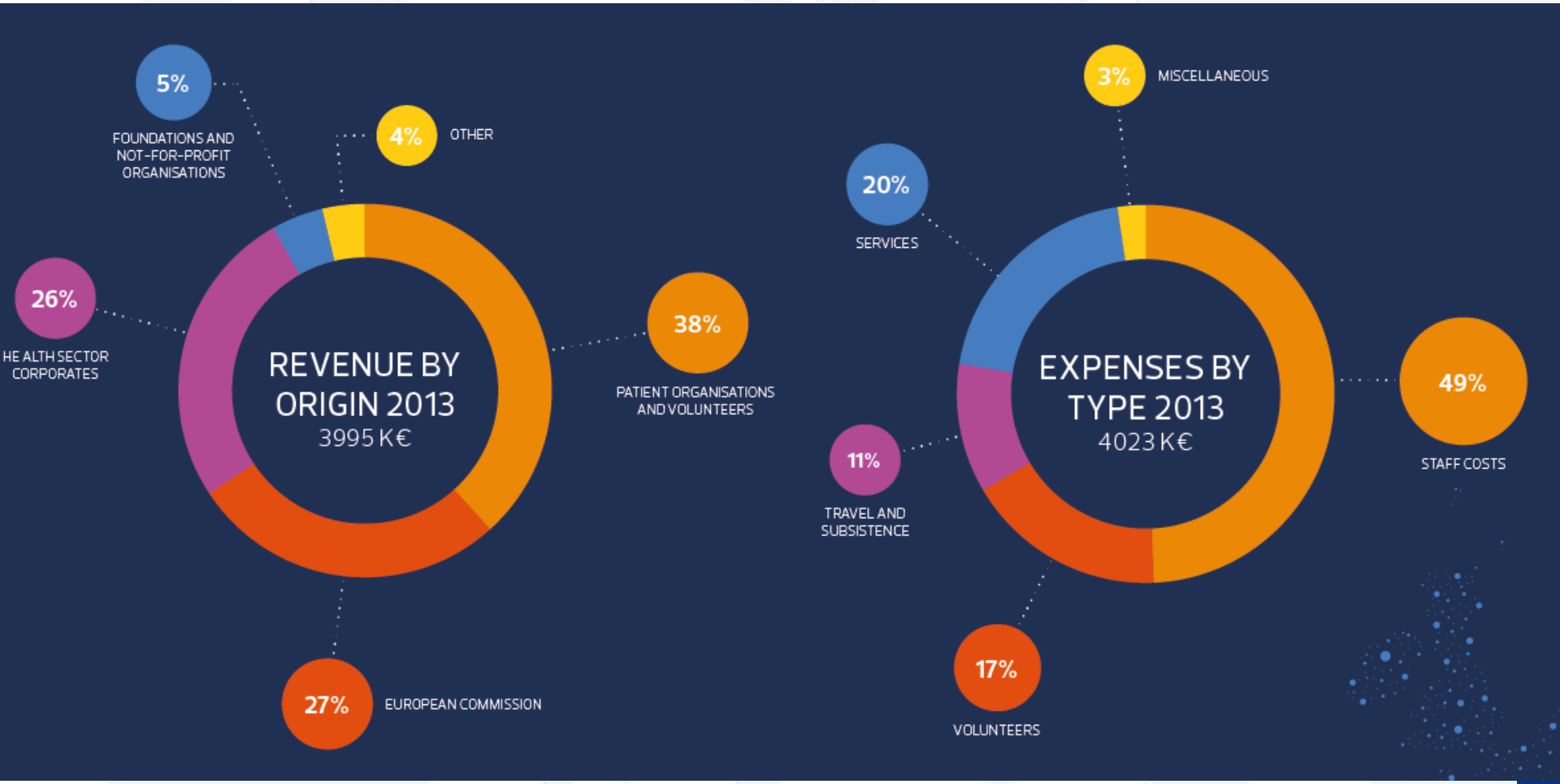
Our Mission

- To build a strong **pan-European community** of patient organisations and people living with rare diseases
- To be **their voice** at the European level
- To directly or indirectly fight against the impact of rare diseases on their lives

EURORDIS in brief

- Founded in 1997
- 633 member patient organisations
- 59 countries (26 EU countries)
- 33 National Alliances of RD Patients Organisations
- 44 European Federations of specific rare diseases
- Outreach to over 1800 patient groups
- Over 4,000 rare diseases represented
- 30 staff
- Offices in Paris, Brussels, London, Barcelona, Geneva
- 260 Volunteers: 55 patient advocates and 205 moderators
- 4 Million € Budget

EURORDIS: Revenues and Expenses



EURORDIS

Major EU policies on rare diseases from the patient perspective

Major Advocacy Achievements
Contribution to EU medicines regulations

EU Regulation on Orphan Medicinal Products, 1999

- Creation of the **COMP - Committee for Orphan Medicinal Products at the EMA** including **3** patients' representatives for the first time
- To date, over 1331 Orphan Drugs Designated
- 93 Orphan Drugs received a MA since 2000; 106 therapeutic indications benefiting approximately 3 million EU patients
- Out of 93 orphan drugs, 20 have lost their Orphan Drugs status after 10 years of EU market exclusivity or are no longer on the market

EU Regulation on Medicinal Products for Paediatric Use, 2006

- Creation of the **CAT - Committee for Advanced Therapies** at the EMA including 2 patients' representatives and their alternates

EU Regulation on Advanced Therapy Medicinal Products, 2007

- Creation of the **CAT - Committee for Advanced Therapies** at the EMA including 2 patients' representatives and their alternates

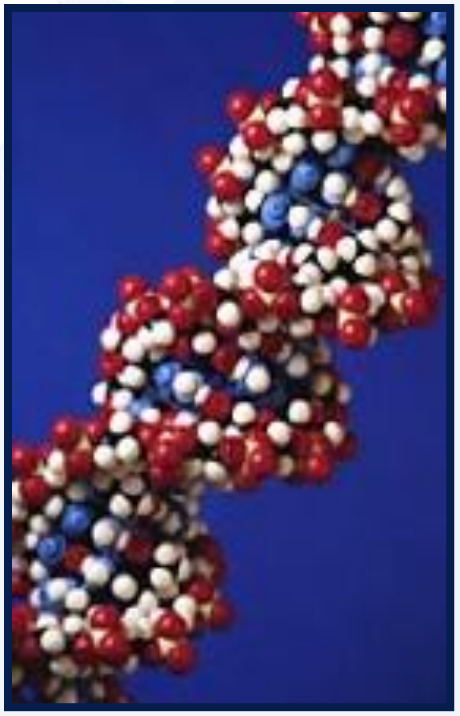
Major Advocacy Achievements
Shaping EU Rare Disease Policy

- **Communication from the European Commission** to the European Parliament, the Council, the European Economic and Social Committee and the Committee of the Regions : « **Rare Diseases: Europe's Challenge » 11 November 2008**
  - First comprehensive policy text addressing issues faced by RD patients, from research on RDs, to diagnosis, access to care and adapted services and development of training, education, awareness on RDs
- **Council Recommendation on an Action in the field of Rare Diseases, 8 June 2009**
  - Adopted by EU Ministers of Health
  - **Promote the adoption of RD National Plan/Strategy in EU MS**
  - European Commission Expert Group on Rare Diseases (*Advisory group to the EC*)

Recent Advocacy Achievements
Contribution to EU Directives/ Regulations

- **EU Directive on Patients' Rights in Cross-border Healthcare in 2011**
  - Article 13 specifically on Rare Diseases
  - Provides the legal framework for European Reference Networks
  - Provides the legal framework for the mobility of patients across EU
- **Most recent contributions from EURORDIS to:**
  - **The revision of the EU Directive on Clinical Trials 2001/20/EC: The Directive has become a EU Regulation** *(adopted by the Council of the European Union in May 2014)*
  - **The proposal from the EC for a EU Regulation on the protection of individuals with regard to the processing of personal data** – *proposal is currently undergoing the legislative process*

Involved in EMA Committees



- **COMP**: Committee for Orphan Medicinal Products
  - 2 EURORDIS representatives (Vice-Chair + 1 full member) + 1 Observer
- **PDCO**: Paediatric Committee
  - 2 EURORDIS representatives (full member & alternate)
- **CAT**: Committee for Advanced Therapies
  - 2 EURORDIS representatives (full member & alternate)
- **PCWP**: Patients' and Consumers' Working Party
  - 2 EURORDIS representatives (full members)
- **Scientific Advice & Protocol Assistance**
- **CHMP**: Committee for Human Medicinal Products

Involved in Commission Expert Group on Rare Diseases (former EUCERD)

- Brings together European main decision-makers in the field of Rare Diseases
- **Objective**: Assist the Commission in the drawing up of legal instruments and policy documents, including guidelines and recommendations, in the field of rare diseases
- **Representation**: 28 EU Member States + Iceland, Norway, Switzerland + EC, EMA COMP + industry + academia + individual experts + patients' representatives

All 8 patients' representatives → members of EURORDIS. They cover main rare disease patient support groups and different European regions. They coordinate advocacy work through regular contacts.

Contribution to the 5 Recommendations EUCERD (Nov. 2009 – July 2013)

- **Quality Criteria for Centres of Expertise for Rare Diseases in Member States**, October 2011
- **Rare Disease European Reference Networks** (RD ERNs), January 2013
- **Clinical Added Value of Orphan Medicinal Products (CAVOMP) Information Flow**, September 2012
- **Rare Disease Patient Registration and Data Collection**, June 2013
- **Core Indicators for Rare Disease National Plans/ Strategies**, June 2013

Upcoming CERD Recommendations (2014 – 2016)

- **Codification of Rare Diseases:** common strategic approach and implementation
- **Rare Disease European Reference Networks** (RD ERNs) : guidance for implementation
- **Genetic testing & counselling services**
- **New Born Screening**
- **Guidelines on Diagnostics & Care for Rare Diseases:** guiding methodological principles
- **Integration of rare diseases into social policies and specialised social services**

Involved in Commission Expert Group on Cancer Control (1st meeting on 23-24 Sept.)

- Brings together European main decision-makers in the field of Cancers
- **Objective**: Assist the Commission in the drawing up of legal instruments and policy documents, including guidelines and recommendations, in the field of cancers, including rare cancers
- **Representation**: 28 EU Member States + Iceland, Norway, Switzerland + EC + industry + academia / cancer prevention + patients' representatives
- 6 patients' representatives → 2 members of EURORDIS

→ they represent rare cancer patient support groups & different European regions. They coordinate advocacy work together with the EURORDIS group of volunteers on rare cancers.

Involved in shaping RD National Plans

EUROPLAN National Conferences 2012-2015

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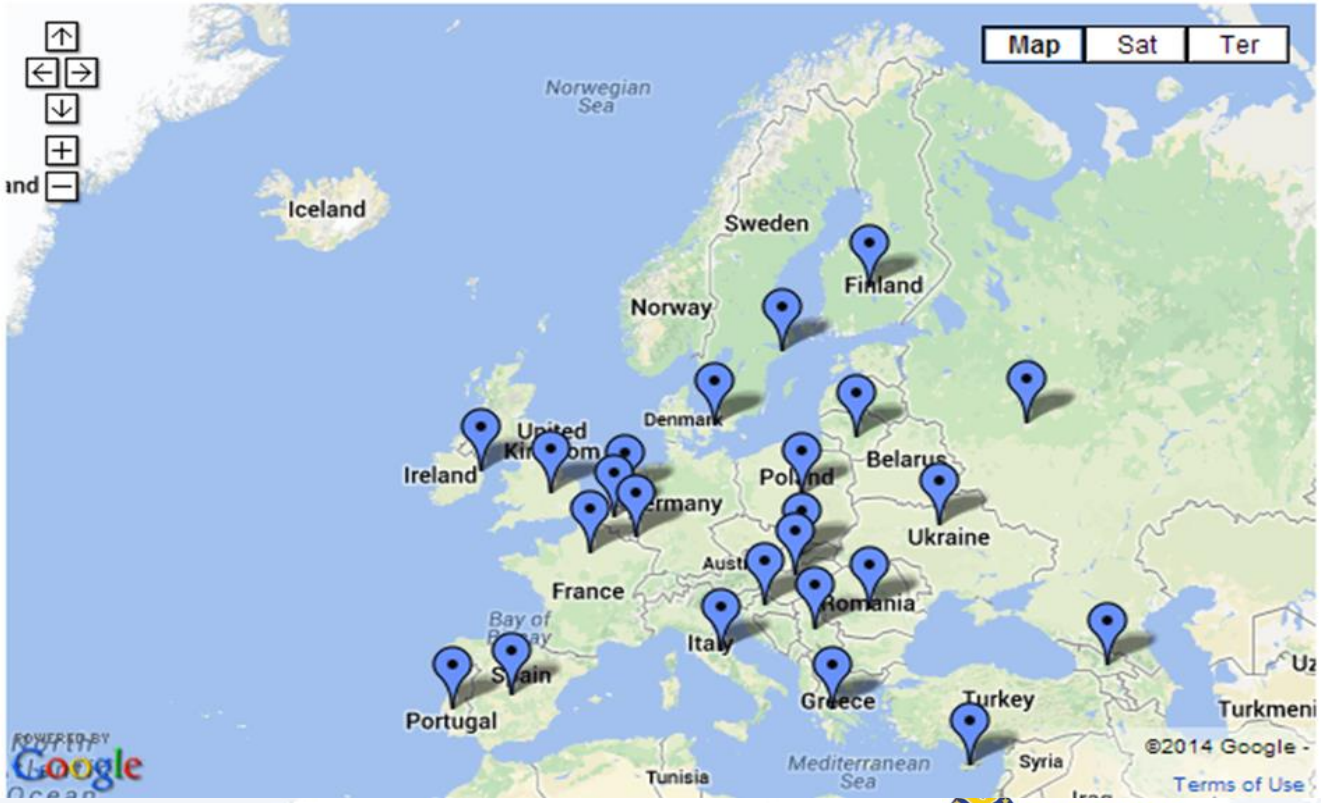
On this map you can find when and where the EUROPLAN National Conferences are being held and details about the organisers, in most cases National Alliances (NA) of Rare Disease patient organisations.

- Final report of each EUROPLAN National Conference



Current Affairs & Strategic Agenda

- The implementation and consolidation of **national strategies / plan in a comprehensive, integrated and sustainable way**, based on the EU (Commission & MSs) priorities (research, Centres of Expertise & European Reference Networks, registries & data collection, good practices of diagnostic & care, codification, information, EU gathering of expertise & common assessments, patients empowerment)
- The development of **European Reference Networks for Rare Diseases per group of diseases articulated with integrated Research Infrastructures** (Biobanks, Registries & Data Collection, -omics, eHealth & Electronic Patient Record, EU Collaborative Clinical Trials)
- The innovative R&D and business model: **adaptive pathways to drug development for rare diseases to derisk it, increase success rate and to place it on an EU wide market**

EURORDIS

in the context of international initiatives

on Rare Diseases

The globalization of rare diseases

- Several RD policies & Regulations are in place
- Development of National Plans / Strategies worldwide
- Increased investments in RD Research : IRDiRC, increased budget of the US NIH or EU dedicated to RD Research…
- Increased investments from industry with market exclusivity incentives provided in several legislations (US Orphan Drug Act, EU Regulation (EC) 141/2000, ….)
- Rare Diseases are often regarded as research models for more common diseases
- Increased collaboration amongst international medicines agencies
- Development of international platforms for RD registries

An International perspective

- Rare Diseases: expertise scarce and scattered

  ⇒ Need to bring together :
  a critical mass of patients
  & medical experts, scientists
  & public health authorities

- In the 21st century, new opportunities from translational research, innovative regulatory science, and information technologies :

  ⇒ Bring more innovative treatments to patients

  ⇒ Increased access and flow of information

  ⇒ Create networking opportunities globally, encourage partnerships

8th European Conference on Rare Diseases & Orphan Products - May 2016, Edinburgh, Scotland

Organised by EURORDIS

Target: +700 participants from all parts of the world

International Rare Disease Day

- Created and coordinated annually by EURORDIS
- Every year since 2008
- Held on the last day of February each year
- Participation in over 80 countries in 2014
- An occasion to raise public and policy-maker awareness for rare diseases
- Annual Policy Event in Brussels
- www.rarediseaseday.org

Rare Connect.org

www.rareconnect.org

- Launched in 2010
- Free-access online Rare Diseases global patient communities
- Over 65 communities involving 564 patients' organisations
- Supported by 3 full-time staff including 2 community managers and 242 volunteer moderators
- Translators offer free translations between 5 languages (English, French, German, Italian, Spanish)

Partnership with European and worldwide umbrella patient organisations

- Strong network with 26 Rare Diseases National Alliances of patients in Europe, including outside EU in Russia, Georgia and Serbia.
- Specific partnerships with the US National Organisation for Rare Disorders (NORD), the Canadian Organisation for Rare Disorders (CORD) and the Japanese Patients Association (JPA): collaborating along same common objectives
- Current regular liaison with RD National Alliances in Russia, Australia, Mainland China, Taiwan, India and Brazil

Launch of Rare Diseases International

- **What is Rare Diseases International (RDI)?**

  An initiative aiming to create an informal network of rare disease patient organisations to form a **global alliance** representing patients & families of all nationalities across all rare diseases.

- **Who is involved?**

  RDI is a EURORDIS initiative with National Alliances around the world with whom we have signed partnership agreements (MoUs).

- **Why Rare Diseases International?**
  - To enhance capacities of *Rare Diseases International* members through information, exchange, networking, mutual support, joint actions
  - To represent its members & people living with rare diseases internationally
  - To promote RDs as an **International Public Health & Research priority** through public awareness and policy

IRDiRC – International Rare Diseases Research Consortium

Objectives:

- 200 new therapies for RDs by 2020
- Means to diagnose most rare diseases by 2020

A consortium to develop a common global vision for rare disease research and to coordinate strategies of funders through the production of policy and recommendations

Involved in IRDiRC – International Rare Disease Research Consortium

- EURORDIS is a partner of the Consortium
- IRDIRC Executive Committee: 1 EURORDIS representative
- Therapies Scientific Committee: Yann Le Cam, Elected Chair
- Therapies Scientific Committee Working Groups (WG) include patients' representatives/ members of EURORDIS
  - WG on Chemically-derived products including repurposing
  - WG on Biotechnology-derived products including cell- & gene-based therapies
  - WG on Orphan drug-development and regulatory processes

EURORDIS OBJECTIVES

To achieve the quickest access to
as many safe, efficient
and affordable medicines
with a real therapeutic added value,
for all rare disease patients
in the European Union

Toward a new sustainable business model for innovative Rare Disease therapies

- **Times are changing**: Economic pressure & Demographic pressure on healthcare budgets / RD scientific opportunities from translational research & Stratified therapies / Growing investors expectations / Society sustainability & values
- **The current business model of OMPs is not sustainable**
- An evolution not a revolution + risks of not acting now
- **Look at essential & long term common interest at stake** across patients, across companies, across competent authorities, rather than antagonising the short term & short take diverging interest
- **Corporate responsibility & leadership & policy innovation**

5 KEY CONCEPTS

- RD Treatments Evidence Generation is a Continuum
- Flexibility of Regulators should become an Official Policy
- Focus on Effectiveness beyond Quality, Safety and Efficacy
- Bridging the Gap Between EU Centralised Regulatory Decision and National Decisions on Pricing & Reimbursement
- Enhancing the Dialogue Between all Stakeholders all Along the Product Development & Life Cycle

10 MAIN PROPOSALS

1. Early Dialogue / scoping / de-risking : EMA + HTA + Payers + PO + Experts
2. RD Data Collection & Registries & Natural History Studies
3. Clinical Trials : EU Expert Opinion + adaptive design & statistical methodology + alternative to animal models + surrogate endpoints
4. Progressive Patient Access / Adaptive Licensing
5. Stronger FDA – EMA Collaboration : Common Guidelines
6. EMA & HTA dialogue: Scientific Advice/ Protocol Assistance
7. HTA EU Common Assessment + Adaptive Core Value Dossier
8. MOCA: Payers dialogue / Value Framework/ Price negotiations
9. Pan-European Managed Entry Agreements & Joint Procurements
10. European Reference Price + Differential Pricing

+ National Measures in RD National Plans/ Strategies

THANK YOU !