




PATIENTS' EMPOWERMENT

Yann Le Cam, Chief Executive Officer

Dia Mundial de les Malalties Minoritàries, 28 February,
CCCB, Barcelona



Speak with one voice
Find solutions to common problems

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
- 615 member patient organisations
- 58 countries (26 EU countries)
- 33 National Alliances of RD Patients Organisations
- 40 European Federations
- Outreach to over 1800 patient groups
- Over 4,000 rare diseases represented
- 29 staff members, offices in Paris, Brussels, Barcelona
- 300 volunteers



EMPOWERMENT

Breaking isolation
Creating a critical mass



Learning from each other
Working together

Community Building

- 615 members in 58 countries
- Council of 33 National Alliances (including Canada, USA & LA)
→ Common Goals, National Plans, Rare Disease Day
- Council of 40 European Federations and Networks
→ Cross border health care, accelerated procedure for multi-centred clinical trials for rare diseases, European Reference Networks of Centres of Expertise, Registries
- Organisation of annual Membership Meetings
→ Barcelona 2002, Namur 2003, Cork 2004, Venice 2005, Berlin 2006, Paris 2007, Copenhagen 2008, Athens 2009, Krakow 2010, Amsterdam 2011, Brussels 2012, Dubrovnik 2013, Berlin 2014
- Organisation of the European Conference on Rare Diseases
→ Copenhagen 2001, Paris 2003, Luxembourg 2005, Lisbon 2007, Krakow 2010, Brussels 2012, Berlin 2014

European Network of Rare Disease National Alliances

- Composed of 33 National Alliances of Rare Disease Patient Organisations
- Governed by the Council of National Alliances
- Fostering the visibility and recognition of National Alliances for Rare Diseases
- Enhancing EURORDIS' outreach to local RD patient groups
- Enhancing EURORDIS and National Alliance cooperation in key policy areas: Common Goals, National Plans, Rare Disease Day
- Capacity building, Empowering and Networking

European Network of European Rare Disease Federations

- Composed of 40 EURORDIS member European Federations
- Representing the disease-specific level
- Enhancing EURORDIS and the Federations capacity in priority policy areas:
European Reference Networks, European research projects, therapy development, web communities, information helplines, Cross-border Healthcare
- Complementing the Council of National Alliances by:
 - Enhancing EURORDIS' outreach to local RD patient groups to build a pan-European RD community
 - Capacity building, Empowering and Networking

EURORDIS Membership Meeting

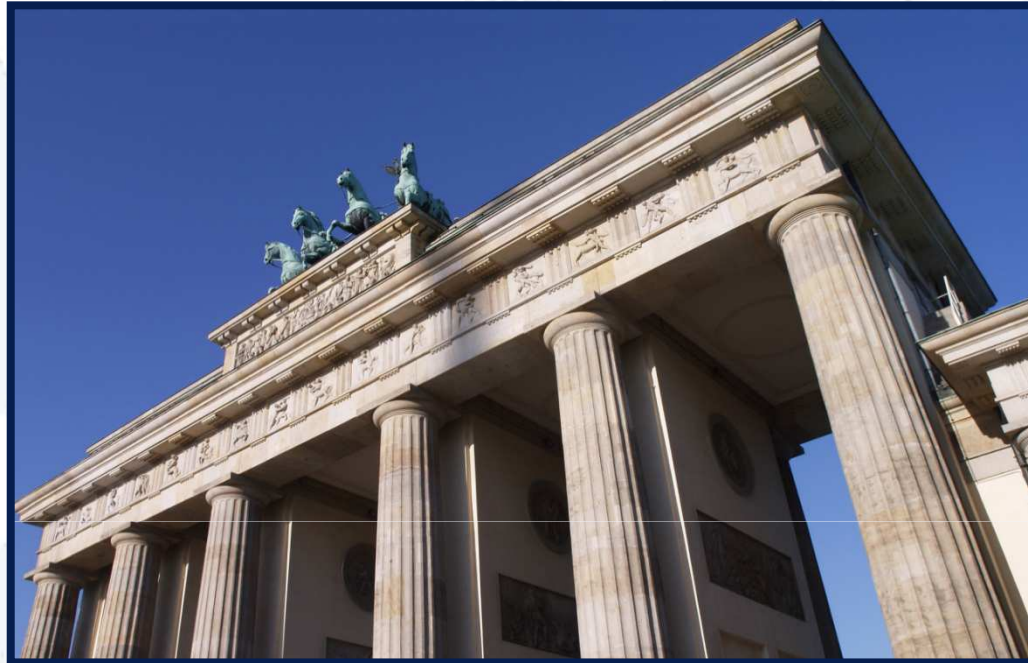
A capacity building annual event for rare disease advocates since 2002

- Past locations include: Barcelona (2002), Namur (2003), Cork (2004), Venice (2005), Berlin (2006), Paris (2007), Copenhagen (2008), Athens (2009), Krakow (2010), Amsterdam (2011), Brussels (2012), Dubrovnik (2013)
- EURORDIS Membership Meeting 2014
Berlin 8 May, 2014
- An opportunity for EURORDIS Members to:
 - Build capacities via targeted workshops
 - Share experiences
 - Network
 - Be up-to-date in main policy and emerging issues

6th European Conference on Rare Diseases & Orphan Products - **ECRD** - 23-25 May 2012, Brussels:

- @700 participants from 55 countries (1/2 patient reps + doctors, researchers, policymakers and industry)
- 110 session chairs / speakers / panelists (25 from EUCERD)
- 38 sessions covering 7 Conference Themes (health policy, research, drug development)
- 4 pre-conference tutorials + satellite meetings
- Posters accepted & presented: 166
- Simultaneous interpretation available: English, Dutch, French, German, Spanish and Russian
- www.rare-diseases.eu





7th European Conference on Rare Diseases & Orphan
Products - **ECRD 2014 Berlin** -
8–10 May 2014, Berlin, Germany

Save the date!

EMPOWERMENT

Advocating for RD patients



Bringing patients at the heart of
European rare disease
policy and regulatory framework

Major Advocacy Achievements

Contribution to EU drug regulations

- EU Regulation on Orphan Medicinal Products in 1999
 - Creation of the **COMP - Committee for Orphan Medicinal Products at the European Medicines Agency** (including 3 patients' representatives for the first time)
 - To date, over 1100 Orphan Drugs Designated + over 82 Products Approved, benefiting approximately 3 million EU patients
- EU Regulation on Medicinal Products for Paediatric Use in 2006
 - Creation of the **PDCO - Committee for Paediatric Drugs at the European Medicines Agency** (including 3 patients' representatives and their alternates)
- EU Regulation on Advanced Therapy Medicinal Products in 2007
 - Creation of the **CAT - Committee for Advanced Therapies** at the European Medicines Agency (including 2 patients' representatives and their alternates)

Major Advocacy Achievements

Contribution to EU Directives/ Regulations

- EU Directive on Patients' Rights in Cross-border Healthcare in 2011
 - Provides the legal framework for European Reference Networks
 - Article 13 specifically on Rare Diseases
- Ongoing contribution from EURORDIS to:
 - **The ongoing revision of the EU Directive on Clinical Trials 2001/20/EC** – Proposal from the EC for a EU Regulation on CTs
 - **The proposal from the EC for a EU Regulation on the protection of individuals with regard to the processing of personal data** – revision of the proposal is currently under review by the Committee LIBE of the European Parliament

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 all issues faced by RD patients, from research on RDs, to diagnosis, access to care and adapted services and development of training, education 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**
 - Creation of the European Union Committee of Experts on Rare Diseases (EUCERD) in November 2009

Major Advocacy Achievements

Promote RDs in European Programmes

- Since 2000, EURORDIS has advocated for rare diseases to be included in the research and health programmes of the European Commission.

- New programmes:

Research programme « **Horizon 2020** » - **2014-2020**

Innovative Medicines Initiatives – IMI 2 – 2014-2020

Health for Growth – 2014-2020

EMPOWERMENT

Informing
Building capacities



Collecting data

Generating patient knowledge

Information & Networking

- Website in 7 languages: English, French, German, Italian, Portuguese, Russian, Spanish
- Weekly eNews in 7 languages
- Brochures, Fact Sheets, Position Papers, Publications
- Blog www.rarediseaseblogs.net
- Rare Disease Day www.rarediseaseday.org
- EURORDIS TV www.eurordis.org/tv
- Online Patient Communities
- Rare!Together
- European Network of Rare Disease Help Lines

EURORDIS Website

- www.eurordis.org
- Information in 7 languages:
 - rare diseases - patient organisations - orphan medicinal products – patient experience and testimonials - EU policies - national plans for rare diseases - research - genetic testing - newborn screening - Centres of Expertise - European Reference Networks – registries - specialised social services - help lines – online patient communities, videos, conferences and other events - EURORDIS activities, and much more

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

- www.rareconnect.org
- Launched in 2010
- Free-access online rare disease patient communities
- Over 42 communities involving over 400 patient associations and over 8000 members
- Supported by full-time community managers and volunteer moderators
- Translators offer free translations between 5 languages (English, French, German, Italian, Spanish)





Understand

Living with a rare disease



Meet

Connect and discuss with other patients



Learn

Get information and find resources

Welcome!

Hosted by trusted patient advocates, this is a place where rare disease patients can connect with others globally. Come in and learn more >

Follow Us Share This



JOIN NOW

It's Free!

Our Communities



Find an online community for your area of interest. Meet, interact and learn from your peers and medical experts. We have 45 rare disease communities with more to come soon!

SELECT YOUR COMMUNITY

Join RareConnect



Meet and interact with others who share your condition in a supportive and friendly environment. Find the information and support you need and share your own experiences.

JOIN NOW

Member Story



Recently diagnosed with FCAS - I'm looking for help with treatment options, particularly if people have... Read more >

Events



Rally for Medical Research Capitol Hill Day - Join th... More >



NBIA Family Conference 2014 - Mark your calendars now... More >



3rd National Pulmonary Hypertension (PH) Conference -... More >

EURORDIS Summer School

- Since 2008
- For Patient advocates
- 4-day annual training on:
 - Clinical Trials
 - Medicines Development
 - EU Regulatory Processes
- Location: Barcelona, Spain
- To date: 190 patient advocates trained from 35 countries and 70 diseases
- Many alumni involved in regulatory processes at the EMA and/or as volunteers in EURORDIS' activities





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Information, Training

through EURORDIS Summer School, Membership Meetings, ECRDs and other fora **have reinforced the capacity of our patients' advocates**

Rare Diseases' patients representatives are members of European and national high-level decision committees

Involved in Commission Expert Group on Rare Diseases (ex-EUCERD)

- Bring 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** → are members of EURORDIS. Cover main rare disease patient support groups and different European regions. They coordinate advocacy work through regular internal meetings.

Contribution to the 5 EUCERD Recommendations

- 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
- Recommendations on Rare Disease Patient Registration and Data Collection, June 2013
- Core Indicators for Rare Disease National Plans/ Strategies, June 2013

Involved in EMA Committees / IRDiRC

- **COMP: Committee for Orphan Medicinal Products**
2 EURORDIS representatives + 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
- *Scientific Advice & Protocol Assistance*
- **IRDiRC: International Rare Disease Research Consortium (EC + NIH + partnering countries + patient organisations)**
Objective: Deliver 200 new ODs and diagnostic test for most RDs by 2020



Involved in Steering Committee of EUROPLAN National conferences

- Objective: aim at developing and sustaining National Plan/Strategy for Rare Diseases
- EUROPLAN conferences are driven by National Alliances of RDs, members of EURORDIS, in 24 countries

Belgium	Greece	Netherlands	Sweden
Croatia	Hungary	Poland	United Kingdom
Cyprus	Ireland	Portugal	Georgia
Denmark	Italy	Romania	Russia
Finland	Lithuania	Slovakia	Serbia
France	Luxembourg	Spain	Ukraine

EMPOWERMENT

RD Patients' Organisations Contributing to Research on RDs



Empowering the patients with rare diseases

The EURORDIS Survey « Role of Patient Groups in Research and their Priorities for the Future » showed that:

- ➔ **Patient Organisations play an important role as catalysts of research**
- ➔ **Patient Organisations provide two types of support research:**
 - **Financial**
 - **Non-Financial:** natural go-betweens for scientists from various fields from the more basic research to therapeutic applications, crucial support in clinical trials

Types of financial support

37% of POs who fund research

77% Initiating and financing a specific research project

75% Co-financing the operating budget of a specific research project

54% Financing the acquisition of a specific research equipment (centrifuge, computer, etc)

47% Financing a fellowship for a young researcher

39% Co-financing meetings of researchers clinicians

30% Co-financing training of researchers / clinicians

What kind of research do patients fund?

81 % Basic research

57% Therapeutics

56% Diagnosis

54% Epidemiology / Natural history of the disease

46% Human and Social Science

24% Assistance technologies / Daily life

19% Research infrastructures

Non-financial support: from clinical trials to institutions

76% Actions aiming at creating links between patients, researchers and physicians

57% Helping to identify patients to participate in clinical trials

49% Providing information and counseling for potential participants in clinical trials

48% Defining research projects by highlighting patients' needs and expectations

45% Collaboration in clinical trials design

30% Participation in scientific committees within institutions

28% Launching campaigns for the collection of biological samples from patients

Empowering the patients with rare diseases



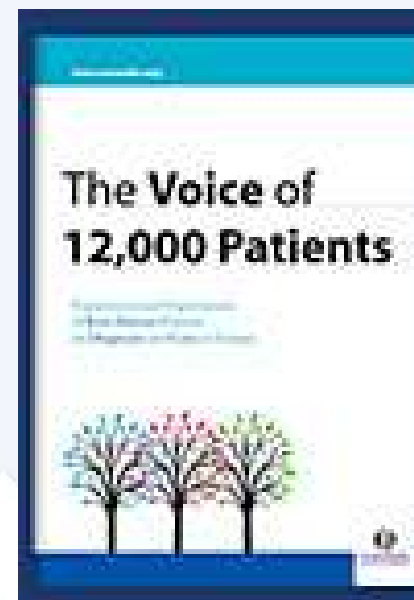
Patient representatives & organisations can:

- Participate in Research networks:
 - EURORDIS participates in Treat NMD, Clinigene, ECRIN
 - Alström UK participates in EuroWabb
 - Cystic Fibrosis Europe participates in ECORN CF...
- Help shape Research Policy
 - EURORDIS promotes RD in EU research policy and funding schemes - Position Paper: *“Why Rare Disease Research”, “Which Priorities for Rare Disease Research?”*
- Raise awareness of the needs of Rare Disease Patients

Empowering the patients with rare diseases

The EurordisCare Surveys 2 & 3 on Access to Diagnosis and Healthcare Services for RD patients in Europe

- 2 surveys conducted through patient organisations; translations by volunteers
- 1st survey: sample of 8 rare diseases; 16 countries
- 2nd survey: sample of 16 rare diseases; 23 countries
- 6 years of work – Sept. 2003 to April 2008
- Results published in the book
- **Use for National advocacy purposes across Europe and beyond**



Empowering the patients with rare diseases

The EURORDIS – EPIRARE Survey on Policy Scenarios for Patient Rare Disease Registries showed that :

➔ **3000 patients and parents participated in the survey**

➔ **Leading to the publication of: “The Voice of 3000 patients” in November 2013**

In addition:

- EURORDIS–NORD-CORD Joint Declaration of 10 key principles for Rare Disease Patient Registries
- “Core Recommendations on Rare Disease Patient Registration and Data Collection” from the EU Committee of Experts on Rare Diseases (EUCERD)

EMPOWERMENT



Better informed patients
Contribute to their own care

Empowered & better informed patients contribute to their care

- Patients and their carers/families are “partners” in the treatment delivery, 24/24, 7/7, year long, life long
- Co-decision makers with the healthcare professionals
- Families have a role as “health care providers”

Empowered & better informed patients contribute to their care

- Life-long conditions, patients need to be empowered to understand their care needs
- Frequently simple principles underpin interventions which may extend life expectancy and enhance quality of life
- Overarching aim of medical management is to anticipate and manage problems to facilitate participation in normal life

Empowered & better informed patients contribute to their care

- **Therapeutic Education Programmes:** aim to facilitate patients to acquire skills in order to understand better their conditions and treatments.
- **Standard Guidelines for Diagnosis and Care:** Must involve the RD patients → example: Danish Tuberos Sclerosis Complex association participates in the national care guidelines
- **Patient data and registries:** patients contribute their data through **informed** consent and **patients reported data**

EURORDIS Board of Directors

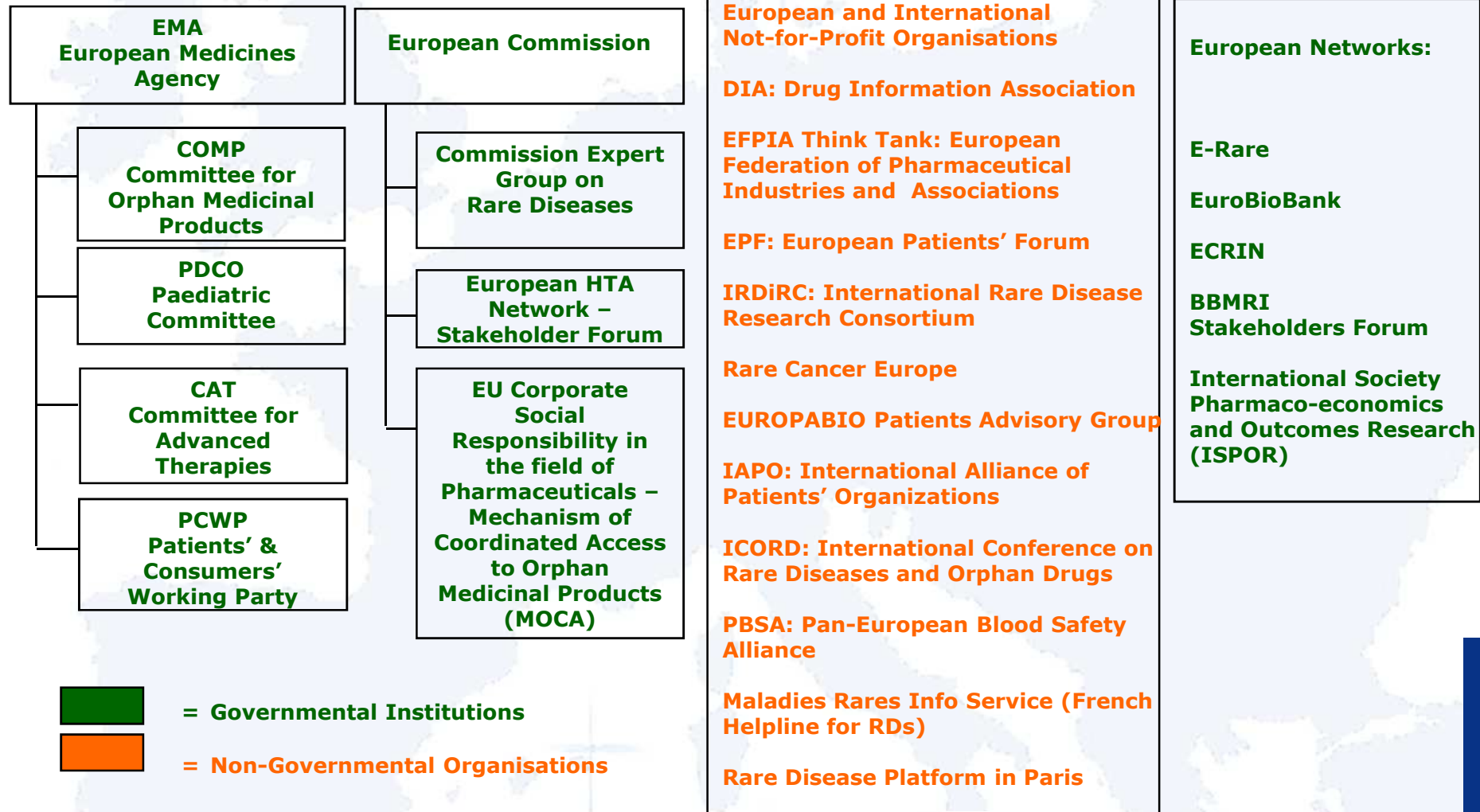


EURORDIS Staff

EURORDIS Governance Chart

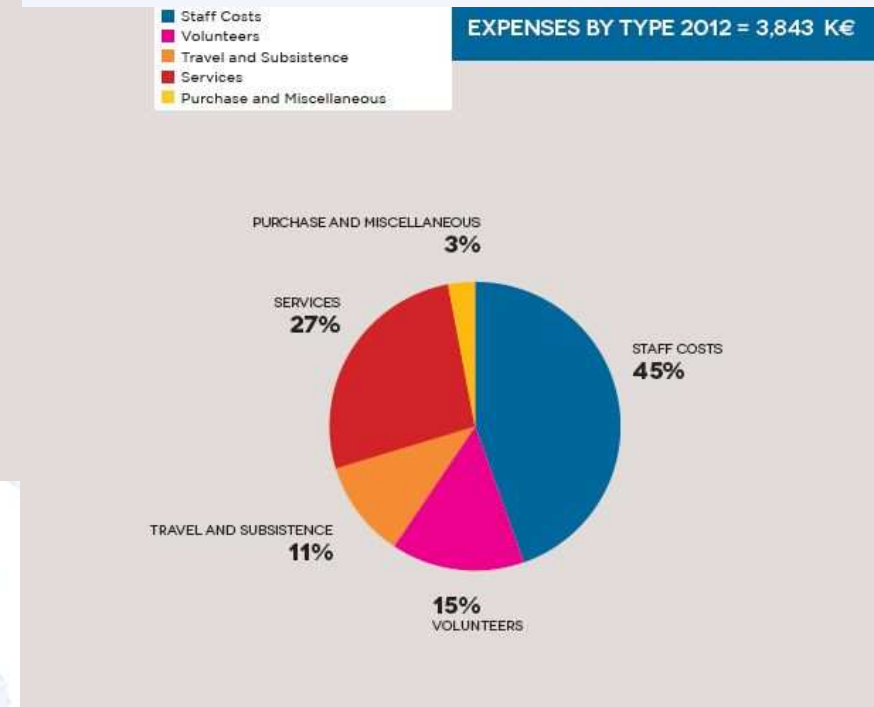
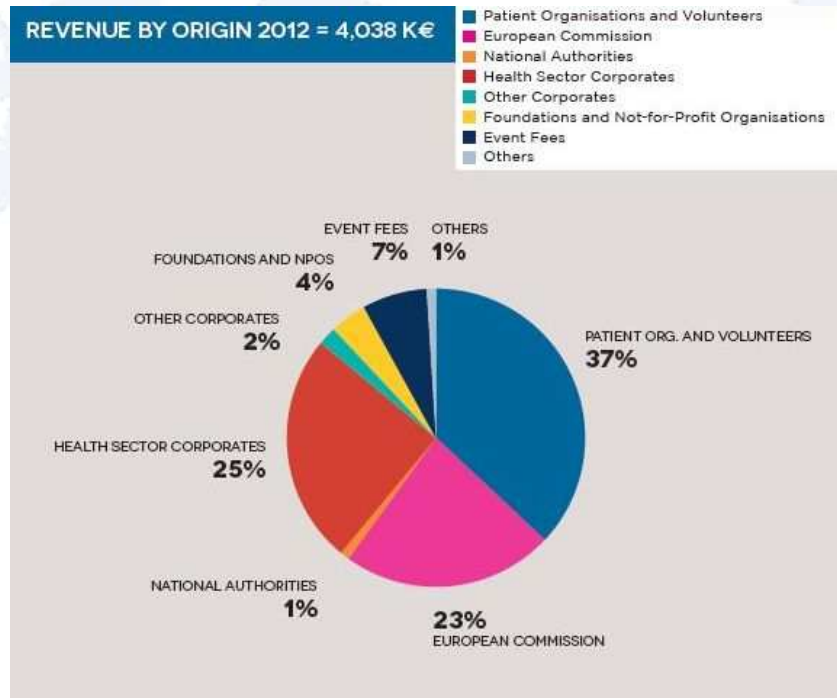



Representation in external organisations & networks



EURORDIS sources of funding

Revenues and Expenses





Working together
and learning from each other

Thank you!