



EURORDIS
Rare Diseases Europe

European
Organisation
for Rare Diseases

ACTIVITY REPORT ²⁰¹³

 WORKPLAN 2014

|| The Voice of Rare Disease
Patients in Europe ||
eurordis.org







EURORDIS
Rare Diseases Europe

TABLE OF CONTENTS

02	FOREWORD	
04	EURORDIS IN BRIEF	
05	STRATEGIC APPROACH 2010–2015	
08	ACTIVITY REPORT 2013	
	HIGHLIGHTS 2013	09
	ADVOCACY	10
	INFORMATION & NETWORKING	20
	HEALTH POLICY & HEALTH CARE SERVICES	31
	RESEARCH, DRUGS & THERAPIES	32
	GOVERNANCE, ORGANISATION & FUNDING	41
	REVENUES & EXPENSES	44
	BOARD OF DIRECTORS	46
	MEMBERS	47
	CONFERENCES	53
	ACKNOWLEDGEMENTS	56
62	WORKPLAN 2014	
	ACTION PLAN 2014	64
	BUDGET 2014	76
	GOVERNANCE CHART 2014	78
	TEAM CHART 2014	80

FOREWORD

BY THE PRESIDENT AND BY THE CHIEF EXECUTIVE OFFICER

2014 began with the celebration of a milestone: EURORDIS reached over 600 members, bringing our membership – at the time of print – to **628 members in 58 countries** (26 EU countries) including **33 National Alliances** and **44 European Federations**. Overall, EURORDIS represents more than 1500 patient organisations throughout the whole of Europe and covers over 4000 different rare diseases. In 2013 alone, **62 new members** joined EURORDIS thus further enhancing its mission of building a strong pan-European community of patient organisations and the 30 million people living with rare diseases in Europe, in order to be their voice at the European level and fight against the impact of rare diseases on their lives.

The **European Network of National Alliances** continued to be strengthened with a new national rare disease alliance created in Poland and more areas of collaboration within the network. Over the course of 2013 the **“Common Goals & Mutual Commitments between EURORDIS & National Alliances in Europe: An Agenda between 2014 & 2020”** was developed and adopted. With this Common Goals & Mutual Commitments initiative, EURORDIS aims to promote greater convergence and collaboration between National Alliances and EURORDIS as much as between National Alliances themselves, in order to further structure and sustain the Rare Diseases Europe movement and to enhance the Voice of People Living with Rare Diseases in addition to the synergies and capacities of their patient advocacy groups for the achievement of common results.

The **EURORDIS website**, with over 300 000 visits per year, was further developed in 2013 to provide a dynamic homepage driven by the weekly **eNews**, greater visibility to our Members' events, a greater visual impact, and a more prominent presence of Social Media. New website sections such as **Pharmacovigilance**, **EURORDIS Awards** and the **EURORDIS Photo Contest** were created while other sections were redesigned and updated. Further sections will be launched in 2014.

Two major information services were launched in 2013: **EURORDIS TV** to provide a dedicated, EURORDIS branded, video portal that aggregates quality rare disease video content; and **EURORDIS InfoHub** to promote access to quality rare disease web-based information services for rare disease patient advocates, patients and families.

Rare Disease Day continued to grow internationally with **72 participating countries in 2013 and 84 in 2014**. The media outreach was strong, with hundreds of news articles collected and over 1000 events organised all over the world. A **new RDD website** was developed and launched this year to improve user friendliness and accessibility. Rare Disease Day has now become an essential leverage to raise public awareness and promote national policies.

The EURORDIS Online Patients' Communities, **RareConnect**, experienced unprecedented growth in 2013. By the end of 2013, 49 disease-specific online communities had been successfully created. These communities saw international partnerships emerge from **462 different patient organisations committed to this international networking**, building cross-border disease knowledge, and reaching isolated people living with the disease. **EURORDIS will scale up this platform in 2014** and expand its utility, creating more online rare disease communities, adding topic groups as a new feature, extending translations from 5 to 7 languages, developing new functions, and exploring partnerships, in particular with academic researchers.



Yann Le Cam / Chief Executive Office

In 2010, EURORDIS made a long-term commitment to the development of **National Rare Disease Plans/Strategies throughout Europe and beyond** and this remains one of our main and foremost key advocacy priorities for 2014. EURORDIS and the **National Alliances continued to organise national conferences in EU Member States and across Europe** throughout 2013 in order to **promote good national measures and a dialogue between all stakeholders**. The theme of the **EURORDIS Membership Meeting 2013 Dubrovnik** was the elaboration of and participation in national plans for rare diseases and the Membership Meeting was dedicated to **building capacities of patient advocates in national plans and strategies and learning from each other** (this was also the case with the EURORDIS Membership Meetings 2010 Athens, 2011 Amsterdam, 2012 Brussels).

As these plans/strategies develop, the **advocacy activities become more technical and require refocusing, while enhancing an exchange of best measures across Europe**. EURORDIS' active participation in the new **European Commission Expert Group on Rare Diseases** (succeeding the European Union Committee of Experts on Rare Diseases) with its **EURORDIS Policy Action Group** will be essential in reaching that goal.

Monitoring the **State of the Art of Rare Disease** policies, developing **Recommendations & the follow-up on their implementation** are two challenges for 2014 for Centres of Expertise, European Reference Networks, Cross-border Healthcare, registries, access to orphan drugs, coding of rare diseases, social policy and services, as well as national plan indicators. Monitoring the implementation of the **EU Directive on Patients' Rights on Cross-border Healthcare**, supporting the development of an **EU Platform on Rare Disease Registries**, participating in new **long-term infrastructure projects** on registries, biobanks, and -omics, and supporting clinical research are other areas EURORDIS will pursue.

The **European Conference for Rare Diseases & Orphan Products – ECRD 2014 Berlin** is part of a broader strategy to push toward concrete implementation of the new EU rare disease policy framework both at European and national levels across the European continent. It is expected to be a milestone in 2014 by bringing to the surface the many achievements of the dynamic multi-stakeholder rare disease community in Europe while sounding the drum of future policy priorities.

Lack of equitable access to orphan medicines across Europe continues to be a high concern aggravated by the impact of the financial and economic crisis. **Earlier, Wider, Equitable, Sustainable Access to Medicines are thus key words for 2014** and EURORDIS is implicated in various initiatives for improving access to medicinal products and services, including plans to promote and participate in dialogue on Patients' Progressive Access and Medicine Alternative Development Pathways, and contributing to pilots for Adaptive Licencing, as well as for Common HTA Assessment Reports and for the Mechanism of Coordinated Access (MoCA) to orphan medicines so **to promote Member State collaboration around the value for new rare disease therapies**. Similarly, EURORDIS will promote European collaboration on pricing based on value, volume and post-Marketing Authorisation data generation, also exploring Managed Entry Agreement and Equitable Pricing mechanisms for improving access. An **Access Campaign** will be launched in 2014 to assess the difficulties patients currently experience in getting medicines and medical care and to promote targeted solutions.

The European Medicines Agency's Committee for Orphan Medicines (COMP) held its **150th meeting** in 2013. EURORDIS has actively participated in the designation of over **1200 orphan medicines to date**. In 2013 **EURORDIS continued its active support to the participation of patient representatives in the Committees and Working Parties of the European Medicines Agency (EMA) – (COMP, PDCO, CAT, PCWP, SAWP, HCPWP), cumulating 138**

days of meetings and 413 scientific dossiers examined for scientific advice/protocol assistance, paediatric studies, and overall clinical development up to marketing authorisation.

In 2013, two new important steps were taken to further engage rare disease patients in policy shaping and decision-making process: beyond the already active engagement of EURORDIS in the International Rare Disease Research Consortium (IRDiRC), **a EURORDIS representative was elected Chair of the IRDiRC's Therapeutic Scientific Committee to reach the goal of 200 new orphan medicines by 2020; while another EURORDIS representative was elected Co-Chair of the Stakeholder Forum of the European HTA Network as well as co-leader of other related Working Groups on Patient Reported Outcomes in partnership with relevant learned societies.**



Terkel Andersen / President

Rare diseases are an international public health challenge and international policy convergence and collaboration can enhance European activities in research, healthcare organisation, information and therapy development. In 2013, EURORDIS resumed the work towards launching Rare Diseases International by disseminating a survey on the international situation of the rare disease field. All respondents are overwhelmingly in favour of EURORDIS initiating **Rare Diseases International as an informal global network of rare disease patient organisations to be launched in 2014 so to be able to speak with one voice**. Turning rare diseases into a global movement and launching Rare Diseases International is a current priority for EURORDIS.

EURORDIS was able to grow in 2013 in spite of a challenging economic environment thanks to actions to diversify its public funding and new initiatives in fund raising. We believe **our organic growth reflects the positive resonance our actions have within the rare disease community**. Still, funding remains a critical issue when it comes to sustaining our development. EURORDIS will continue to diversify and consolidate its resources in 2014.

Our community is governed by common values and modes of action. We believe in solidarity and equitability, social justice and fairness. We work through patients' empowerment and capacity building of patient advocates, exchange and mutual support, gathering of experience and networking, partnership with all stakeholders. We are audacious and innovative. We proactively collect facts and data to support the promotion of policy. We trust that facts and words shape policy and actions which result in better outcomes. We know what we want for patients and believe that a patient driven agenda has the capacity to catalyse all interested parties and support public policy.

EURORDIS IN BRIEF

EURORDIS, is a non-governmental patient-driven alliance of patient organisations and individuals active in the field of rare diseases, dedicated to improving the quality of life of all people living with rare diseases in Europe.

EURORDIS was founded in 1997 by four patient groups from different therapeutic fields: the Association Française contre les Myopathies (AFM), Vaincre la Mucoviscidose, Ligue nationale contre le Cancer (LNCC), and AIDES Fédération.

Today it is supported by its members and by the Association Française contre les Myopathies, AFM – Téléthon, the European Commission, corporate foundations and the health industry.

EURORDIS is the voice of 30 million people affected by rare diseases throughout Europe.

KEY FIGURES 2013



EURORDIS' 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, and – directly or indirectly – to fight against the impact of rare diseases on their lives.





STRATEGIC APPROACH 2010–2015



EURORDIS in 2015 has consolidated its position as the organisation of reference for rare diseases in Europe and is recognised as an actor in worldwide processes having impacts on patients living with rare diseases in Europe

- Rare Diseases as a Public Health priority in Europe (8th EU Framework Programme for Research & Technology, 3rd EU Public Health Programme, other policy legislations and programmes)
- Being the voice of all rare diseases, genetic or not, including rare cancers, and very rare diseases, open to Europe at large (48 countries)
- Raising rare disease awareness amongst general public (in particular the International Rare Disease Day and European Year of Rare Diseases campaign)
- Rare diseases become an international movement and gain visibility and influence in international instances (Council of Europe, WHO & UN)
- Production, sharing and accessibility of patient-generated knowledge



EURORDIS in 2015 is facilitating the effective implementation of European regulations and strategies at national levels in more policy areas for the benefit of patients and families

- A Public Health priority in European countries (Member States and beyond)
- National Plans in each Member State with patient-centered approaches incl. Centres of Expertise, research, medicines, registries, information, social services. Monitor their implementation including indicators.
- Development / Consolidation of European Networks integrative of European and National levels:
 - European Reference Networks of Centres of Expertise
 - European Research Networks and European Research Infrastructure for rare diseases
 - European Network of Information Help Lines
- Adjust actions on the basis of feedback from PO members on the effective implementation of rare disease regulations and policies (evaluation process) and remaining unmet needs (research budget, Centres of Expertise, Best Clinical Practice on Diagnostics and Care, Quality of Life)
- A special focus on research. The role of EURORDIS shall aim at:
 - A higher public awareness in support of rare disease research
 - A more favourable research policy framework for rare diseases
 - A development of EURORDIS' interactions with the research community and learned societies

- A promotion of the development of European Research Networks and European Research Infrastructure for rare diseases
- A promotion of the participation of patients in research and therapeutic developments – which enhances capacity building of patient representatives
- A promotion of the participation of patient representatives in ethical committees in clinical research and human genetics
- A special focus on information and quality of life. The role of EURORDIS shall aim at being a direct operator in the following fields:
 - Rare disease-specific help lines in national languages linked in a European Network
 - Patient-based knowledge, generated and shared in care, cope and quality of life in a holistic approach
 - Educational information on the management of specific symptoms which are common to across different rare diseases (ex. hyperactivity, sleep disorders, etc.)
- Educational information on managing the impact of rare diseases on family life (effect on parents, siblings, integration at school, empowerment of young adults, aging etc.)
- A special focus on information and quality of life. The role of EURORDIS shall aim at enhancing and catalysing actions in the following fields by the means of partnerships:
 - Production and availability of educational material and courses for health care professionals, social workers, etc
 - Production and availability of validated and updated Information on Respite Care Services (RCS), Therapeutic Recreation Programmes (TRP), Resource Centres (RC) and other rehabilitation services such as Adapted Housing (AH).
 - Promotion of research on quality of life in EU framework programmes in research and public health



EURORDIS in 2015 has developed enriched and more supportive capacity building relationships with its members and empowerment of volunteers

- Maintain a high level of legitimacy and credibility by maintaining a high level of consent amongst EURORDIS' members
- An integrative (in main areas of activities) and supportive volunteer programme well recognised inside and outside
- Intensify capacity-building and networking with and between the National Rare Diseases Alliances and European Rare Diseases Federations for improved efficacy and decentralization
- Member patient organisations as key relays to their families and patients to generate and access relevant customised information
- Capacity building networking, sharing experience and common tools, giving an easy access to good practices to empower patient advocates



EURORDIS in 2015 is more sustainable in terms of human, financial and organisational resources and governance

- Values and governance: Well established shared Values and governance processes
- Leadership sustainability of the Board
- Decreased and better-balanced workload inside the staff, more high level volunteers, efficient balance of workload between staff / volunteers
- Web communications central in strategy / organisation / work process
- Financial sustainability: Attract more public funding, diversify sources of revenues (Corporate other than pharmaceutical or medical device companies, Foundations), generate own unrestricted resources (Gala dinner, donations). Consolidate administrative process and budget control.
- Integrative IT infrastructure, database and tools

COMMUNITY STRUCTURE OVERVIEW

- | National Alliances on Rare Diseases
- | European Rare Diseases Federations or Networks
- | Patient Organisations' Members other than above
- | Patient Organisations and Patient Outreach in All of Europe
- | RareConnect
- | Committees, Task Forces, Group

STRATEGIC PARTNERSHIPS

- | AFM : Association Française Contre les Myopathies
- | NORD: US National Organization for Rare Disorders
- | CORD: Canadian Organization for Rare Disorders
- | ORPHANET: The web server of medical experts generated and validated information
- | JPA: Japan Patients' Association
- | RPU: Russia Patients' Union
- | INDUSTRY through EURORDIS Round Table of Companies and other initiatives
- | ACADEMIA for education & capacity building and for social & policy research



ACTIVITY REPORT 2013

HIGHLIGHTS 2013	09
ADVOCACY	10
INFORMATION & NETWORKING	20
HEALTH POLICY & HEALTH CARE SERVICES	31
RESEARCH, DRUGS & THERAPIES	32
GOVERNANCE, ORGANISATION & FUNDING	41
REVENUES & EXPENSES	44
BOARD OF DIRECTORS	46
MEMBERS	47
CONFERENCES	53
ACKNOWLEDGEMENTS	56

HIGHLIGHTS 2013

- | EURORDIS celebrated reaching over **600 member patient organisations (606)**, located throughout 56 different countries, including 526 full members in 26 EU countries and representing over 4000 different conditions.
- | **Rare Disease Day 2013** was organised in collaboration with partners in 71 countries worldwide, with over 1000 related events taking place. A Rare Disease Day European Policy Event was organised in Brussels and co-hosted by two MEPs. A brand new website was launched in November for Rare Disease Day 2014.
- | **RareConnect** continued to expand with 14 new online patient communities launched in 2013 reaching 49 global communities in collaboration with 462 patient organisations and 8500 members.
- | 2 new information services were launched in 2013: **EURORDIS TV** to provide a dedicated, EURORDIS branded, video portal that aggregates quality rare disease video content; and **EURORDIS InfoHub** to promote access to quality rare disease web-based information services for rare disease patient advocates, patients and families.
- | EURORDIS continued its support to developing **National Plans and Strategies for rare diseases** in Europe, with National Alliances, within Work Package 4 of the EUCERD Joint Action (EUROPLAN) facilitating the ongoing organisation of 24 National Conferences including content guidelines, indicators as well as patient advocate capacity building, exchange and networking. In 2013, 13 EUROPLAN National Conferences were organised by EURORDIS National Alliances.
- | **EUCERD Recommendations** adopted in 2013 on: European Reference Networks for Rare Diseases (RD ERNs); Rare Disease Patient Registration and Data Collection; Core Indicators for Rare Disease National Plans/Strategies
- | 8 patient representatives (4 full and 4 alternate) were appointed to the newly established **Commission Expert Group on Rare Diseases** (replacing the EU Committee of Experts on Rare Diseases – EUCERD)
- | The **6th EURORDIS Summer School** for patient advocates was held in Barcelona in collaboration with ECRIN-IA, for the second year in a row. 2013 gathered 37 participants representing 15 countries and 39 different diseases. EURORDIS also continued its strong involvement with the European Patients' Academy on Therapeutic Innovation project (EUPATI).
- | **413 dossiers on orphan drugs**, advanced therapies and paediatric investigation plans were reviewed as part of participation in the European Medicine's Agency Scientific Committees. These include: 195 dossiers for orphan drug designations, 20 classification or certifications by the CAT and 198 Paediatric Investigation Plans by the PDCO
- | The **15th Workshop of the Council of National Alliances** (33 National Alliances for Rare Diseases) was held, for the second time in a row, in conjunction with the **6th Workshop of the Council of European Federations** (44 rare diseases-specific networks)
- | The "**Common Goals & Mutual Commitments between EURORDIS & National Alliances in Europe: An Agenda between 2014 & 2020**" was developed and adopted by the EURORDIS Board of Directors and the Council of National Alliances. This initiative aims to promote greater convergence and collaboration between National Alliances and EURORDIS as much as between National Alliances themselves.
- | EURORDIS strengthened its presence in the governing bodies of the **International Rare Disease Research Consortium (IRDiRC)**: a EURORDIS representative was unanimously elected Chair of IRDiRC's Therapeutic Scientific Committee and EURORDIS representatives continued to be present in IRDiRC Working Groups.
- | EURORDIS resumed the work towards launching **Rare Diseases International** as an informal global network of rare disease patient organisations, by disseminating a survey on the international situation of the rare disease field. All respondents were overwhelmingly favourable of the initiative, to be launched in 2014.
- | Memoranda of Understanding (MoUs) were signed with the **Canadian Organisation of Rare Disorders (CORD)** in late 2012 and with the **Japan Patient Association (JPA)** in May 2013 to promote rare diseases as an international health priority.



ADVOCACY

Sustaining Rare Diseases as an EU Public Health Priority

.....

EU COMMITTEE OF EXPERTS ON RARE DISEASES (EUCERD)

The European Union Committee of Experts on Rare Diseases was formally established via the European Commission Decision of 30 November 2009 (2009/872/EC). This committee was charged with aiding the European Commission with the preparation and implementation of Community activities in the field of rare diseases, in cooperation and consultation with the specialised bodies in Member States, the relevant European authorities in the fields of research and public health action and other relevant stakeholders acting in the field.

The seven¹ patient representatives to the EUCERD proposed by EURORDIS and appointed by the European Commission in 2010 continued their advocacy and advisory role to EU officials on behalf of the rare disease

community in 2013, and participated in the following EUCERD plenary meetings:

- 31 January – 1 February, 7th EUCERD Meeting, EC, Luxembourg
- 5 – 6 June, 8th EUCERD Meeting, EC, Luxembourg

In addition to contributing to the EUCERD Recommendations adopted, the patient representatives of the EUCERD were also particularly active in their advisory role to the European Commission regarding: National Plans for Rare Diseases; Rare Disease Day and the European Conference on Rare Diseases and Orphan Products; Integration of rare diseases into several policies and social services.

All reports and recommendations produced by the EUCERD in 2013 are available on www.eucerd.eu

EUCERD ACHIEVEMENTS:

The mandate of the EUCERD came to an end in July 2013. During its 3-year mandate, the EUCERD adopted five important Recommendations and one opinion aimed at harmonising rare disease policies across EU countries in the following areas:

- EUCERD Recommendations on Quality Criteria for Centres of Expertise for Rare Diseases in Member States, 24 October 2011
- EUCERD Recommendations on "Improving Informed Decisions based on the Clinical Added Value of Orphan Medicinal Products (CAVOMP) Information Flow", September 2012

- EUCERD Recommendations on European Reference Networks for Rare Diseases (RD ERNs), 30 January 2013.
- EUCERD Core Recommendations on Rare Disease Patient Registration and Data Collection, 5 June 2013
- EUCERD Recommendations on Core Indicators for Rare Disease National Plans/Strategies, 6 June 2013
- New Born Screening in Europe: Opinion of the EUCERD on Potential Areas for European Collaboration, July 2013

¹ Eight patient representatives were appointed to the EUCERD in 2010, amongst them Torben Grønnebak (Rare Disorders Denmark) who sadly passed away in 2012

THE EU PUBLIC HEALTH PROGRAMME 2014–2020: "HEALTH FOR GROWTH"

On 26 February 2014, the European Parliament adopted the Commission proposal for the establishment of the third Health Programme 2014–2020: "Health for Growth".

Rare diseases are recognised as a priority. Since 2010, EURORDIS has conducted intense advocacy activities and in 2012 worked with MEPs to table amendments to enhance the rare diseases priority.

THE EU RESEARCH PROGRAMME 2014–2020: "HORIZON 2020"

In November 2013, the European Parliament adopted the Carvalho Report on the Implementation of the Regulation for the establishment of Horizon 2020, the new EU Research Framework Programme for 2014–2020. MEPs carried the amendments originally proposed by EURORDIS, thus making the original Commission proposal on Horizon 2020 more aligned with EURORDIS' priorities on rare disease research, as well as with the IRDiRC² objectives. The legislative framework for Horizon 2020 was finally adopted in December 2013 with the positive vote of the Council of the EU. The day after, the European Commission published the Work Programme for 2014 and 2015 including its related calls.

In view of the adoption of the programme, EURORDIS was in contact with the European Commission, DG Research and Innovation, and other stakeholders to have insights on the first calls for proposals.

EURORDIS was therefore able to prepare a training and information session for the Council of the European Federations addressing: 1) RD research in EC Research Framework Programmes from FP5 to FP7; and 2) opportunities for RD research in Horizon 2020. On the latter, the session aimed to describe the new specific elements of the upcoming seven-year funding programme, to provide the first insights on the Work Programme 2014–15 ahead of the official publication of the first calls. The session was conceived to support the research activities of member POs with relevant

information and tools facilitating the applications of patient groups for the EC research funding programme.

EURORDIS also has anticipated the launch of a 3rd ERA-Net on rare disease, eRare and the calls on research infrastructures.

RARE DISEASE DAY EURORDIS POLICY EVENT

Since 2008, EURORDIS has organised an annual Policy Event in Brussels on the occasion of Rare Disease Day that raises awareness and moves forward key policies and initiatives benefiting people living a rare disease.

Keeping with this tradition, EURORDIS organised a Policy Event in Brussels for Rare Disease Day 2013. The Policy Event on the topic of "Faster Access to Medicines for Rare Disease Patients – The Transparency Directive" was held in Brussels on 26 February. It was a major achievement to have two MEPs, Antonya Parvanova and Christian Busoi, co-host the event, demonstrating the success of EURORDIS' advocacy actions. There were 100 participants on-site and the event was watched by 600 viewers on the EURORDIS website including 217 viewers watching live from 31 countries.



Support to National Plans for Rare Diseases in Europe

In 2013, EURORDIS continued its support to developing National Plans and Strategies for rare diseases in Europe within the Work Package 4 of the EUCERD Joint Action: "Support for the implementation of plans or strategies at MS level" (EUROPLAN). This project started in March 2012 and will end in August 2015. EURORDIS has the specific task of coordinating and facilitating the organisation of 20 EUROPLAN National Conferences in the European Union as well as 4 other national conferences in European countries outside the EU, namely Georgia, Russia, Serbia and Ukraine.

EUROPLAN NATIONAL CONFERENCES: A POWERFUL TOOL TO FOSTER NATIONAL PLANS/STRATEGIES

The EUROPLAN National Conferences are patient-led, organised by National Alliances and involve all relevant stakeholders including policy-makers, researchers, physicians and other healthcare professionals, industry and patients' representatives.



A team of 10 EURORDIS-EUROPLAN Advisors, all from National Alliances, cooperate with EURORDIS staff to support the organisation of 2 to 3 National Conferences each.

The EUROPLAN National Conferences apply the same format and methodology in order to address

throughout the conference the themes developed in the **“Recommendation on an action in the field of rare diseases”** of the Council of the European Union: Governance of plan/strategy; classification and coding; research on rare diseases; access to care (including orphan medicines)/ Centres of Expertise/ European Reference Networks; sustainability; patient empowerment; gathering of expertise.

These themes are addressed in the workshops of the EUROPLAN conference. **“Content guidelines”** have been developed for each workshop in order to provide background information on relevant legal and policy texts and specific questions of the discussion. EURORDIS staff, with the support of EURORDIS-EUROPLAN-Advisors, analysed the material and compiled 6 guidance documents that were finalised in early 2013. Three of them were updated in summer 2013 to include new EUCERD Recommendations.

As a result of an in-depth preparation by the National Alliances and EURORDIS, the EUROPLAN National Conferences are being recognised as the best forum to: address key issues relevant to improving care and the quality of life of patients living with rare diseases; gather the opinions of the different stakeholders; and make concrete proposals to advance policies for rare diseases.

In 2013, 13 EUROPLAN National Conferences were organised by EURORDIS National Alliances⁴.

LIST OF EUROPLAN CONFERENCES 2013:

- SLOVAKIA: SAZCH – Slovak Alliance for Rare Diseases – organised its first EUROPLAN conference on 28 February 2013 in Bratislava.
- ROMANIA: RONARD – Romanian Alliance for Rare Diseases – organised its second EUROPLAN conference on 24–25 May 2013 in Bucharest.
- FINLAND: HARSO – Rare Disease Alliance Finland – organised its first EUROPLAN conference on 21 September 2013 in Helsinki.
- POLAND: ORPHAN – Rare Diseases Poland – organised its second EUROPLAN conference on 27–28 September 2013 in Warsaw.
- HUNGARY: HUFERDIS – Hungarian Alliance for Rare Diseases – organised its second EUROPLAN conference on 25–26 October 2013 in Budapest.
- LITHUANIA: The Lithuanian Society of Human Genetics and the Ministry of Health organised the first EUROPLAN National Conference on Rare Diseases under the auspices of the Lithuanian Presidency of the Council of the EU, on 13–14 November 2013 in Vilnius.
- CYPRUS: CARD – Cyprus Alliance for Rare Disorders – organised its first EUROPLAN conference on 14–15 November 2013 in Nicosia.
- THE NETHERLANDS: VSOP – the Dutch Genetic Alliance – organised its second EUROPLAN conference on 14–15 November 2013 in The Hague.
- LUXEMBOURG: ALAN – Alliance for people living with neuromuscular and rare diseases – organised its first EUROPLAN conference on 19–20 November 2013 in Luxembourg city.

European countries outside the EU:

- RUSSIA: Two patient organisations – Russian Patients Union and Genetica – jointly organised their first EUROPLAN conference on 28 February 2013 in Moscow.
- UKRAINE: CSMA; Kharkiv charitable foundation for children with spinal muscular atrophy – organised the first EUROPLAN conference on 27 March 2013 in Kiev.
- GEORGIA: GeRad – Georgian Foundation for Genetic and Rare Diseases – organised the first EUROPLAN conference on 17 April 2013 in Tbilisi.
- SERBIA: NORBS – Serbian National Organization for Rare Diseases – organised the first EUROPLAN conference on 5–7 December 2013 in Belgrade.

⁴ In Lithuania, as there is not a National Alliance of rare disease patients, the conference was organised by the Vilnius Society of Human Genetics together with the Ministry of Health under the auspices of the Lithuanian Presidency of the European Union.

NATIONAL PLANS IN THE CONTEXT OF EURORDIS MEMBERSHIP MEETING 2013

The 2013 EURORDIS Membership Meeting in Dubrovnik (May 2013) was centred on National Plans for Rare Diseases. Several parallel workshops discussed important issues such as implementing a national plan, funding of a plan, Centres of Expertise and European Reference Networks, registries, strategies and tools for improving patients' access to orphan medicinal products and specialised social services. EURORDIS staff and Advisors prepared the material for the workshops and the plenary session. The discussion that took place in Dubrovnik helped as well for the preparation of EUROPLAN National Conferences.

CORE INDICATORS FOR NATIONAL PLANS AND STRATEGIES ON RD

EURORDIS and its Advisors started in late 2012 a process of selection of core common indicators for RD National Plans from the list of 59 EUROPLAN Indicators (developed in the EUROPLAN project in 2010) to be utilised by Member States for data collection when monitoring the implementation of National Plans or Strategies on Rare Diseases.

This selection of core common indicators led by EURORDIS was crossed over with the Delphi method applied by the EUCERD Joint Action WP4 leader, the Italian Istituto Superiore di Sanità (ISS), who performed a similar selection with representatives of the EU Member States (Ministries of Health). Following a first consultation with the EUCERD (January 2013), a workshop was organised by the ISS (March 2013) to finalise the selection process. As a result of this integrated approach, 21 Core Indicators were selected and submitted to the EUCERD which in June 2013 adopted the list of Core Indicators as EUCERD Recommendations.

Rare Diseases: An International Public Health Priority

RARE DISEASES INTERNATIONAL

*In March 2012, the EURORDIS Board of Directors adopted the orientation to create an informal network **"Rare Diseases International"** to expand the movement of rare diseases at an international level, provide mutual support between patient organisations and be able to speak with one voice.*

In 2013, in consultation with international partners such as CORD and IAPO, EURORDIS developed a survey that was disseminated to over 100 contact/patient organisations internationally. There were 64 respondents from 37 countries around the world replying to a diverse array of questions. All respondents were overwhelmingly in favour of the creation of a Rare Diseases International initiative with 97.62% replying that they would be interested in joining. The results of the international survey will be used to finalise a **"Joint Declaration: Rare Diseases as an International Public Health Priority"** in 2014. In parallel, EURORDIS has also worked on a Rare Diseases International Action Plan for 2014–2016 in order to launch Rare Diseases International in 2014.

EURORDIS–NORD STRATEGIC PARTNERSHIP

For the past 15 years, EURORDIS and the US National Organization for Rare Disorders (NORD) have been joining efforts to improve the lives of rare disease patients on both sides of the Atlantic. In July 2009, a strategic partnership, aimed at converging strategies between the two organisations for the period 2009 – 2015, was signed with each organisation bringing mutual support to the development of a series of activities.



The Strategic Partnership between EURORDIS and NORD continued to play a key role in promoting rare diseases as a public health priority in 2013, with notable areas of collaboration being: Rare Disease Day 2013; EURORDIS' participation in the NORD Corporate Council Meeting in Washington DC, May 2013; EURORDIS' participation in the NORD–DIA US Conference on Rare Diseases and Orphan Products: "The New Era in Health Care" in North Bethesda, USA, October 2013; EURORDIS and NORD continuing the development of their joint rare disease online communities platform, RareConnect; NORD being an official conference partner for the European Conference on Rare Diseases and Orphan Medicinal Products 2014 Berlin.





EURORDIS-CORD PARTNERSHIP

EURORDIS and the Canadian Organization for Rare Disorders (CORD) have been collaborating together for many years. CORD is a member of the EURORDIS Council of National Alliances of rare disease patient organisations. On 19 October 2012, an official partnership was sealed between CORD and EURORDIS to further enhance collaboration on international advocacy activities, especially in the areas of Rare Disease Day, support to National Plans and collaborations on Conferences.

In 2013, CORD and EURORDIS continued their close collaboration with CORD actively participating in Rare Disease Day 2013; EURORDIS taking part in CORD's 5th Rare Disease Day Conference; EURORDIS contributing to CORD's "Consultations Toward A Canadian Plan for Rare Diseases" held in Toronto, Canada, 12 November; EURORDIS participating in CORD's conference held in Ottawa, Canada, 30 September.



EURORDIS-JPA PARTNERSHIP

EURORDIS and the Japan Patients Association (JPA) signed a Memorandum of Understanding on 26 May 2013, bringing patient advocates from Europe and Japan together to promote rare diseases as an international health priority.

Through the signed MoU, the two organisations agreed to collaboration in the following areas: Rare Disease Day; sharing the experience on National Plans for Rare Diseases; advocacy coordination and possible co-signing of Joint Position Papers; promotion of rare diseases as an International Public Health Priority through the creation of Rare Diseases International and the promotion of rare diseases at the WHO; collaboration on conferences.



Improving Access to Orphan Medicines

CLINICAL ADDED VALUE OF ORPHAN MEDICINAL PRODUCTS (CAVOMP-INF)

Disparities in access to market-authorized orphan medicines exist between as well as within the European Member States. This situation translates into varying delays from the marketing authorisation date to the pricing and reimbursement decisions amongst Member States. The CAVOMP-INF process considers how to coordinate at European level the respective procedures for marketing authorisation and for reimbursement decisions.

In 2013 EURORDIS has continued to advocate for the operational implementation of CAVOMP, joining efforts with EFPIA and EMA in dialogue with the Commission and reaching out to payers and HTA bodies. In the meantime essential components of CAVOMP have been addressed under other policy streams such as early dialogue pilots at EMA; parallel scientific advice by EMA and HTA agencies, future common HTA assessment reports within the SEED project.

Discussions are still ongoing on some aspects of the CAVOMP, such as the prevalence of the therapeutic indication of orphan medicines, or the compilation of assessment reports.

MECHANISM OF COORDINATED ACCESS TO ORPHAN MEDICINAL PRODUCTS (MOCA)

EURORDIS has participated in the Mechanism of Coordinated Access to Orphan Medicinal Products (MoCA) of the Platform on Access to Medicines in Europe

since its creation in 2010. This platform is one of the three work areas of the Process on Corporate Responsibility in the field of Pharmaceuticals launched by the European Commission (DG ENTR).

In 2013 EURORDIS followed up closely the beginning of the implementation phase and the launch of the first pilots through the MEDEV, Medicines Evaluation Group, and through advocating directly DG SANCO within the European Commission.

DIFFERENTIAL PRICING

EURORDIS participated in the meetings on the subject of "Equity of Access and Sustainable Pricing Approaches for Pharmaceuticals in Europe", organised by FIPRA International, and chaired by former MEP John Bowis and the Head of the Institut national d'assurance maladie-invalidité (the Belgian National Health & Disability Insurance), Jo De Cock. After a series of meetings, the group adopted a proposal for a Code of Conduct on "differential pricing as a way forward for better access to pharmaceuticals in Europe", in June 2013. More recently, the group agreed that the issue of "looking closely at prices for medicines" will have to be addressed by the new Commission, after the European elections, and that there must be some level of differentiation and transparency introduced in order to reach equity throughout Europe. EURORDIS has been liaising with different stakeholders on how to tackle inequalities of access to medicinal products, within and beyond Europe and has regularly underlined the need to have all stakeholders at the table, especially the still missing ones: payers and HTA bodies.

PATIENT PROGRESSIVE ACCESS

Patient progressive access, also referred to as adaptive licensing, has continuously been promoted by EURORDIS since 2012. EURORDIS position is to optimise the use of current EU Regulatory Framework – Conditional Approval, Exceptional Circumstances, Risk and Benefit Management Plans – to provide earlier access to rare disease therapies in the absence of alternative therapies or when highly innovative treatments may translate in patients' medical benefits. EURORDIS has been, and will continue to be in the coming years, instrumental in the elaboration of the concept and piloting of "adaptive licensing" as part of the overall strategy and efforts aimed at improving access to orphan medicinal products. EURORDIS has regularly liaised with the European Medicines Agency (EMA), HTA bodies and European Commission.

In 2013, EURORDIS organised a Round Table of Companies Workshop on **"Practical aspects of progressive patient access to orphan medicinal products, post-marketing evidence generation & related pricing questions for rare disease patients"**; this workshop brought together 80 participants with high level speakers from EMA, Industry and patient advocates. A joint letter initiated by EURORDIS co-signed with European Patient Forum, EFPIA and the EUROPABIO EBE TF on orphan drugs was sent to the European Commission in December 2013 calling for the concrete launch of the first pilots. As a result of continuous advocacy action, the first possible pilots are envisaged from 2014.



Patients Involvement in the Benefit/Risk Assessment

.....

EURORDIS has long called for the involvement of patients' in risk benefit assessment and for better consideration to patients' relevant outcomes and patient preferred treatment options. Since 2011, EURORDIS has played a front line advocacy role in promoting such involvement, working closely with the patient and consumer working party of the CHMP⁵, starting patient involvement in some scientific advisory groups of the CHMP and training patients for such activities.

In 2013, this advocacy activity has culminated with a presentation by EURORDIS on behalf of the Patients' and Consumers' Working Party to the EMA management board in November. It is expected that first pilots of involving a permanent observer and patient representatives in CHMP will start in 2014 and EURORDIS is advocating for at least a couple of pilots to be on Orphans in the framework of the SEED project (Shaping European Early Dialogues for health technologies).

⁵ Committee for Medicinal Products for Human Use

Improving Access to Quality Care

FOLLOWING-UP THE IMPLEMENTATION OF THE DIRECTIVE ON PATIENTS' RIGHTS IN CROSS-BORDER HEALTHCARE

Directive 2011/24/EU on patients' rights in cross-border healthcare clarifies the rules on access to healthcare in another EU country, including reimbursement. EURORDIS has been instrumental in placing the focus of the Directive on patients' rights in cross-border healthcare on patients living with a rare disease and on the specificities of rare diseases which require mobility of experts and expertise, of data and of patients at some crucial moments. The three main elements of EURORDIS advocacy activity have been reflected in the Articles relating to: 1. Rare Diseases; 2. The European Reference Networks for Rare Diseases; and 3. Cooperation between Member States on Health Technology Assessment.

To help EURORDIS members and National Alliances best advocate for patients' rights for the transposition of the Directive on Cross-border Healthcare, a Questions and Answers document was prepared and translated in 7 languages (Bulgarian, English, French, German, Greek, Italian and Spanish) in 2012. Throughout 2013, National Alliances were encouraged to engage discussion with their health authorities for the national transposition of the Directive and EURORDIS started collecting testimonies from patients on difficulties they are still having.

EURORDIS representatives have been invited on a regular basis to participate in meetings and subjects on this issue, at both EU and national levels, most often as speakers, in order to explain and discuss the concrete consequences for rare diseases patients following the entering into force of the Directive. The aim is both to support individual patients as well as to manage expectations that have been raised too high in some Member States, especially due to disinformation by local media.

EUROPEAN REFERENCE NETWORKS (ERN)

Within the framework of both the EU Directive on Patients' Right to Cross-Border Healthcare and the reflection process that commenced in 2005 on Centres of Expertise and European Reference Networks (ERN), EURORDIS has contributed substantially, from the very beginning, to the development of the process leading to the finalisation of the EUCERD Recommendation on ERN at the end of December 2012 and its adoption in January 2013.

EURORDIS has put forward throughout 2013, a patient-centred vision of the overall system to be achieved at EU level, aimed at establishing a limited number of ERN, covering all rare diseases, gathered by therapeutic areas, pragmatically starting from existing networks of experts and patient groups and widening in a step-wise approach to the most mature centres wishing to establish a reference network at European level. The overarching goal of the whole system is, in EURORDIS' opinion, to reach a global system where no rare disease patient will be left out and each patient will "find a home", after 10 years by the end of the process, including undiagnosed patients.

To this effect, EURORDIS' actions in 2013 have included: Broad dissemination of EUCERD Recommendation on European Reference Networks of Rare Diseases to rare disease patient organisations (website, eNews etc); Organisation of a patient advocates capacity building on CoE in the context of National Plans/Strategies as part of the EURORDIS Membership Meeting 2013 Dubrovnik (Workshop 5) attended by 47 participants; participation to several meetings to give the rare disease patients' perspective; Implementation of Content Guidelines, based on EUCERD Recommendations, for the National Conferences on National Plans supported by EUCERD Joint Actions WP4, so to include main measures for CoE and ERN in National Plans.



Other issues

.....

REVISION OF THE EU CLINICAL TRIALS DIRECTIVE

EURORDIS has been invited to several meetings, conferences and workshops around Europe to present the rare diseases patients' perspective.

In the Regulation as adopted, some of the main EURORDIS proposals for amendments have been kept in the final document. These include: the need to foster clinical research for the development of Orphan Medicinal Products; the in-depth assessment of clinical trials application is of particular importance for CT in rare diseases; assessment of clinical trials applications must be conducted on the basis of appropriate expertise; specific expertise should be considered when assessing clinical trials involving people with rare diseases.

EURORDIS will have to follow up the implementation phase, especially concerning patients' involvement in Ethics Committees and make sure that the reporting Member State will have access to the best available knowledge.

REVISION OF THE DATA PROTECTION DIRECTIVE

The Revision of the Data Protection Directive has been identified as a key issue with high rare disease specificity. EURORDIS has been carrying out an internal reflection process on this revision, in particular in relation to patient registries and mobility of data while protecting patients' privacy.

EURORDIS and EPIRARE (the European Platform for Rare Disease Registries) co-organised a Lunch Debate on Data Protection and Health research, hosted by MEP Marielle Gallo (EPP, France), at the European Parliament in Brussels on 26 September 2013.

The reflection on Data Protection has been complex and it has been difficult to find the right balance between the protection of rare disease patients' data and the "protection" of research efforts. Rare diseases patients find themselves trapped in the following paradox: on the one hand they need research to be fostered and rare data to be exchangeable in a not too cumbersome manner; on the other hand, they are more easily identifiable that patients affected by common diseases and – with the progress in the field of genome sequencing, more data on their genetic profile will be collected leading to greater vulnerability to genetic-based discrimination. This very complex situation has led EURORDIS to acknowledging the need for further internal reflection on how to best represent the interests of rare disease patients.

TRANSPARENCY DIRECTIVE

After having advocated the European Parliament and Commission on the Transparency Directive and having proposed several amendments, EURORDIS dedicated the Policy Event in Brussels at the occasion of the RDD 2013 to the issue of Transparency.

The European Commission adopted a revised proposal of the Transparency Directive based on the amendments of the European Parliament in March 2013. Since then, the proposal is blocked in Council and the discussion on this file is being postponed after the European elections. EURORDIS will monitor this evolution and will take some further action to help advancing this file.

CONFLICT OF INTEREST AT THE EUROPEAN MEDICINES AGENCY (EMA)

Since September 2011, changes in the EMA policy and procedures on conflict of interest occurred on several occasions following evaluation and discussions at the European Parliament.



The EMA organised a workshop entitled "Best expertise vs conflicts of interests: Striking the right balance" on 6 September 2013 where members of the task force participated, and EURORDIS will continue to advocate for the best policy on conflicts of interest, whilst ensuring patients with rare diseases are more and more consulted by the EMA scientific committees, when needed. Issues that remain to be discussed, in particular for rare disease organisations, relate to restrictions that apply when the organisation to which a patient expert is affiliated is funded by a healthcare industry even if the patient in question receives no funds and has never been in contact with the company. The definition of "consultation" when pertaining to the pharmaceutical industry also needs to be clarified.

The EMA management board decided in December 2013 to amend the policy on conflicts of interests, and this will be done in 2014.

PARTNERSHIP WITH THE INTERNATIONAL SOCIETY FOR PHARMACOECONOMICS – ISPOR

Outcome research is rapidly evolving and major changes are already being implemented. One common feature is the agreed consensus on the importance to obtain the opinion of the patients or their representatives in all processes, and also sometimes to collect information from them. The measurement of the impact of medicines in the patient's life is a key component of the reflection on how to best assess the value of medicines for society.

In 2013, EURORDIS became a member of the International Society for Pharmacoeconomics and Outcome Research (ISPOR) and signed an MoU partnership. EURORDIS is chairing the newly created Patient Centred Special Interest Group.

The objectives of the Patient Centred Special Interest Group (PC-SIG) are:

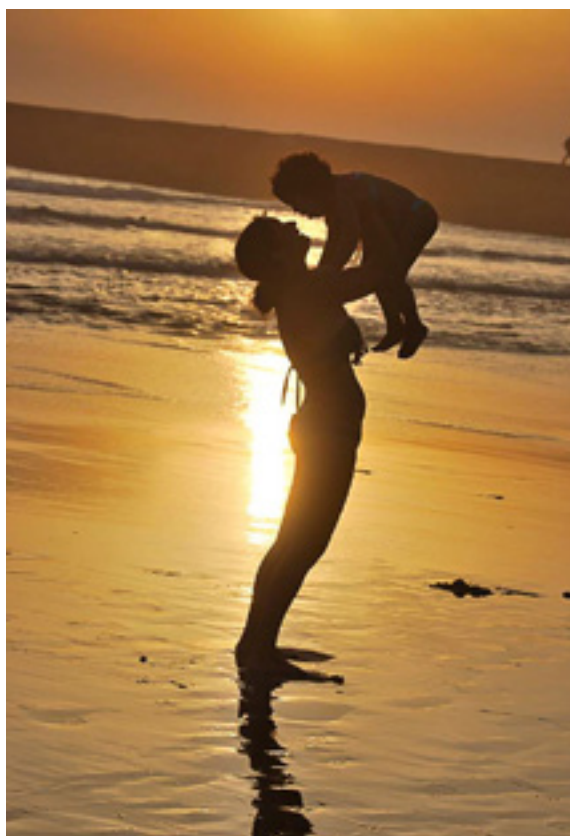
- 1 To determine how to best involve patients and their representatives in all stages of the decision making, on an opinion providing basis, or on a decision making basis, in concordance with the Denver Principles of Patient Advocacy (see reference);
- 2 To analyse expectations and obstacles for a greater involvement of patients in the benefit-risk and the value assessment of medicines;
- 3 To ensure an effective participation of patients' organisations in the creation and development of tools to measure outcomes patients themselves can measure;
- 4 To identify training needs and opportunities for patients and their representatives in the domains of interest.

The PC-SIG met for the first time in Dublin in November 2013 and will soon decide on an action plan.

PARTNERSHIP WITH THE EUROPEAN FEDERATION OF INTERNAL MEDICINE (EFIM) & THE FOUNDATION FOR THE DEVELOPMENT OF INTERNAL MEDICINE IN EUROPE (FDIME)

The European Federation of Internal Medicine (EFIM) is a scientific organisation founded in 1996 to provide a scientific organisation of internal medicine on a European basis. EFIM was formed by bringing together the national societies of internal medicine in European countries, both inside and outside the European Union. EFIM currently comprises 33 member Societies representing over 30,000 internists.

The purpose of EFIM is to re-emphasise the importance of Internal Medicine in patient care in a world of increasing specialisation. Since 1997 EFIM has held scientific congresses every 2 years and annually since 2007. The European Journal of Internal Medicine (EJIM) is the official journal of EFIM. Internal medicine doctors treat 50% of the rare diseases in adults.



EURORDIS, EFIM and FDIME agree in good faith to work together to promote or develop awareness on rare diseases, initiatives aiming at improving access to information, quality diagnosis, treatment and multidisciplinary care, and also research for rare diseases.



INFORMATION & NETWORKING

Membership base – 606 members

.....

65 new members joined EURORDIS in 2013 (34 full members and 22 associate members). New countries represented: Japan, Kazakhstan, Lebanon, Singapore, Uruguay.

At the end of 2013, EURORDIS had 606 members in 56 countries, 36 of which are European countries, 26 being members of the European Union.

EURORDIS Membership Meeting Dubrovnik 2013

.....

Every year EURORDIS organises its Membership Meeting (EMM) in a different European city. This is an occasion for patient representatives to gather and learn from each other. The EURORDIS Membership Meeting comprises the Annual General Assembly, a Conference and several workshops. The majority of participants are EURORDIS member organisations and other patient organisations, but about 25% of the participants represent policy makers, industry and academia. Each Membership Meeting has a specific focus on issues of high importance.

The EMM 2013 Dubrovnik's main theme was the elaboration of and participation in National Plans for Rare Diseases. The Meeting included a plenary session and 12 workshops with topics including: **"Introduction to National Plans", "Funding for National Plans", New-born screening & Genetic testing", "Rare Disease Patient Registries", "European Reference Networks & Centres of Expertise".**

207 participants from 31 countries attended the Membership Meeting, including 27 fellowships which EURORDIS distributed to patient representatives from countries surrounding Croatia

Council of National Alliances

.....

National RD Alliances serve to bring together all the many RD organisations in a particular country. The CNA (Council of National RD Alliances), established by EURORDIS, allows national representatives of rare disease patients to work together on common European actions.

EURORDIS supports a network of 33 National Alliances for Rare Diseases, of which 28 constitute the Council of National Alliances (CNA). One new National Alliance joined the Network in 2013, representing Poland.

The CNA's main activities in 2013 were the work on National Plans for Rare Diseases, notably in the framework of the Joint Action on Rare Diseases as well as the preparation and coordination of Rare Disease Day 2014.

In 2013, two CNA Workshops took place: the first one at the occasion of the EMM 2013 Dubrovnik and the second one, held partly in common with the Council of European Federations (CEF), to allow cross cutting discussions on common issues in Paris in October.

The CNA workshop held in Dubrovnik, which gathered 38 representatives from 24 countries, was the occasion to present National Alliances with the European Year for Rare Diseases initiative. Other topics included Rare Disease Day 2014, Common Goals and Mutual Commitments, as well as the conferences held in the framework of the Europlan project.

The CNA workshop that took place in Paris in October 2013 gathered 33 participants the first day of the

workshop and focused on the following topics: Overview of National Plans for Rare Diseases, National Conferences for Rare Diseases, Rare Disease Day 2014, Common Goals & Mutual Commitments, Rare Diseases International.

On the second day, the meeting was held together with the CEF, and gathered 45 participants to focus on: European Year of Rare Diseases, Access to medicines campaign, Social services.



The **“Common Goals& Mutual Commitments between EURORDIS & National Alliances in Europe: An Agenda between 2014 & 2020”** was developed over the course of 2013 with the active contribution of a dedicated CNA working group. The final document was adopted by the CNA in October 2013 and the EURORDIS Board of Directors in November 2013.

Through the Common Goals & Mutual Commitments, EURORDIS & National Alliances will aim to the best of their ability and in accordance with available resources to:

- Consolidate their position as the organisations of reference for rare diseases at national level and as European Networks and be recognised as actors in worldwide processes having impacts on patients and families living with a rare disease in Europe;
- Consolidate their activities to raise public awareness, in particular the Rare Disease Day and the European Year for Rare Diseases;
- Facilitate the development and the effective implementation of a unique EU integrated, comprehensive and long-term strategy to address

patients' needs everywhere in Europe, driven by patient advocacy, developed through partnership of all stakeholders, and guided by regulations & directives (laws), recommendations & communications (policies), road maps & programmes & guiding principles & expert recommendations (technical guidance);

- Facilitate the development and engage in the effective implementation of national plans & strategies for rare diseases;
- Consolidate their joint policy recommendations and activities in drug development, centres of expertise, European reference networks, biobanks& registries, good clinical practices for diagnosis & care, specialised social services & integration of rare diseases within national social policies, patients' advocates empowerment;
- Strive for and maintain supportive capacity building relationships with their members and empowerment of volunteers; have the objective to become sustainable in terms of human, financial, organisational resources and governance.



Council of European Federations & Networks

.....

European Federations aim to federate national rare disease specific patient organisations at the European level. The CEF (Council of European Federations), established by EURORDIS, allows European Federations to work together on common European actions.

Representatives of European Rare Disease Federations gathered in Paris in October to discuss issues that are important across Europe and across diseases. For the second time, part of the meeting took place in conjunction with the CNA (Council of National Alliances) to discuss cross cutting issues and share experience. This part of the meeting focused on: The European Year of Rare Diseases, Access to medicines campaign, Social services.

The second part of the meeting was dedicated to: Registries, Rare-Bestpractices, RareConnect for European RD Federations.

The next day, the representatives participated in a one-day capacity building session on Research, including presentations on IMI, Horizon 2020 and 2 case studies of research projects initiated and driven by patient organisations: AKUre Consortium and Alliance Sanfilippo.

EURORDIS continued for the fourth year the programme **"Support to European Rare Disease Federations"**. The smallest and/or youngest organisations often have great difficulties in financing their network meetings (Board meetings, Network meetings, conferences, etc). EURORDIS has provided financial support to a total of 24 960 € to 12 European RD Federations to help them organise various meetings.

RARE!TOGETHER

Rare!Together aims at helping in the creation, operation and management of European Rare Disease Federations, in particular through the website raretogether.eurordis.org. This website is continuously updated in order to remain a good practice reference handbook and toolkit for existing European Federations and for patient organisations planning to set up their European Federation.

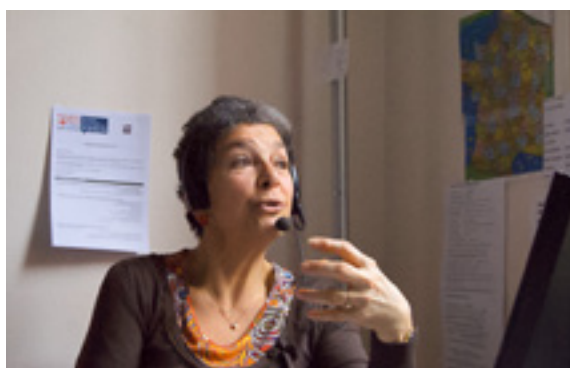
European Network of Rare Disease Help Lines

The European Network of Help Lines for Rare Diseases aims at better serving the needs of the callers by sharing resources, best practices, common tools and knowledge base. It was created in September 2006 and is coordinated by EURORDIS. The network aims at increasing awareness, efficiency, and best practice standards for its members.

In 2013, 12 help lines participated in the activities, from 8 countries: Bulgaria (ICRDOD), Croatia (National Help Line Zagreb), France (Maladies Rares Info Services, AFM-Téléthon), Italy (Coordinating Centre for Rare Diseases Veneto Region, and Telefono Verde Malattie Rare), Portugal (LinhaRara), Romania (NORO, Myasthenia Gravis Romania), Spain (SIO-Feder and ENERCA), Switzerland (Info Maladies Rares, a new help line)

In addition to its annual meeting, the network conducted its 6th Caller Profile Analysis based on all enquiries received in October. A report is available. The network submitted an article on the 5th Caller Profile Analysis to the Interactive Journal of Medical Research (review in progress).

In July 2012, EURORDIS, on behalf of the Network of Rare Diseases Help Lines, wrote to the European Commission DG Connect to reserve a "116" unique call number for rare disease help lines that operate in Europe. However, on 26 June 2013 the network received a negative response from European Commission, due largely to the lack of information by Member State representatives, not to a lack of interest in the proposal.



RareConnect–Rare Disease Online Communities

Launched in 2010 by EURORDIS in partnership with the US National Organization for Rare Disorders (NORD), RareConnect, the online patient community portal rareconnect.org, has helped patients and families connect through an online social network in order to support each other and share vital experiences on aspects of living with a rare disease. Organised into disease-specific communities, this platform also provides links to quality information and involves patient associations in the creation, governance and growth of each community. Additionally, it offers a translation service whereby patients can request a human translation of any forum post into any one of the available languages.

RareConnect experienced unprecedented growth in 2013. By the end of 2013, 49 disease-specific online communities had been successfully created. These communities saw international partnerships emerge from 462 different patient organisations that are committed to international networking, building cross border disease knowledge, and reaching isolated people living with the disease.

During 2013 the RareConnect website received over 400 000 visits from 214 countries which led to 8500 patients or caregivers signing-up as members of RareConnect.

RareConnect is a multilingual platform in five languages (English, French, German, Italian, and Spanish). During 2013, RareConnect translated almost one million words across five languages.

Of the 49 currently live communities, 15 were launched during 2013 for the following diseases:

- Amyloidosis
- Neurodegeneration with Brain Iron Accumulation NBIA
- Cobb syndrome
- Pitt Hopkins syndrome
- Erdheim-Chester Disease
- Porphyria
- FOXP1 gene mutation
- Propionic Acidemia
- Hereditary Leiomyomatosis and Renal Cell Cancer
- Tumor Necrosis Factor Receptor-Associated Periodic Fever syndrome
- Leigh syndrome
- Undiagnosed Brain Diseases
- Lowe syndrome
- Malignant Infantile Osteopetrosis
- Myasthenia Gravis



The RareConnect network is also composed of over 200 moderators who volunteer their efforts and expertise in managing the diseases and finding quality information online. Many of these moderators come from EURORDIS member organisations, and play a key role in requesting, creating, moderating, and maintaining the communities with the goal of enhanced global cooperation and

increased knowledge on daily living with a rare disease. Four capacity-building webinars took place during the year in order to train and empower moderators in the governance and management of a successful online patient community. In May, in Dubrovnik, a workshop was dedicated to the RareConnect project and social media use during the EURORDIS Membership Meeting.



EURORDIS Website

The EURORDIS website provides information relating to the role of patient organisations in the development of rare disease and orphan medicines policy in a patient-friendly language translated into 7 languages (English, French, German, Italian, Portuguese, Russian and Spanish), while also outlining the activities provided by EURORDIS. The total number of website visits over the year amounted to 308 205, a 20% increase compared to 2012.

NEW WEBSITE SECTIONS

New website sections added in 2013 include Pharmacovigilance, EURORDIS Awards, and EURORDIS Photo Contest, while other sections were redesigned and updated such as the EURORDIS Round Table of Companies, European Conference on Rare Diseases and Orphan Medicinal Products, Training Resources and News & Events. In addition new sections on What we do, EU Rare disease policy, Compassionate use and European Year for Rare Diseases were gradually developed in 2013 for launch in 2014.

The new section on Pharmacovigilance entitled: **“Improve the safety of your medicines”** describes actions patients can take to participate in pharmacovigilance (e.g. reporting suspected adverse drug reactions), and how pharmacovigilance is organised at the European and Member State level: information on the “black triangle” for medicines requiring additional monitoring, geographic map of Europe with links to online or paper based reporting tools, a video made by the European Medicines Agency, and a link to EudraVigilance, the European database of side effects reported by patients and healthcare professionals. The Pharmacovigilance website section is available in 7 languages.

This new section was prepared by members of EURORDIS DITA⁶ Task Force: www.eurordis.org/pharmacovigilance.



⁶ Drug Information Transparency and Access (DITA) task force

EURORDIS TV

EURORDIS TV was launched in April 2013. It provides a dedicated, EURORDIS branded, video portal available within eurordis.org that aggregates quality RD video content and promotes videos produced by EURORDIS.

Through this function, video footage is provided of: testimonies, EURORDIS speeches & events, conference presentations, training or capacity building videos, Rare Disease Day events, all through the EURORDIS website.

In addition, EURORDIS TV promotes in house recordings presenting key policy issues / papers / Summer School and EURORDIS Membership Meeting testimonies.

Since its launch, EURORDIS TV has uploaded over 300 videos and has received over 5000 unique visitors.



EURORDIS InfoHub

• • • • •



The internet is one of the first places people living with a rare disease turn to in search of information. Without wishing to duplicate the resources which already exist on the web, EURORDIS has developed the EURORDIS InfoHub for the purpose of meeting three basic needs:

- 1 to provide a guide to patients on how to find quality rare disease information on the internet. This is achieved through a series of short videos including How to optimise your Internet search with Google; Searching databases for rare disease, clinical trial and orphan drug information; Using PubMed, a bibliographic database.
- 2 to provide a place where people can find a quality-driven selection of rare disease resources on the internet. EURORDIS has developed a selection of reliable and helpful websites selected for patients and families searching the internet. This selection has been developed based on criteria officially adopted by EURORDIS.
- 3 to comprise a specific tool which gives access to search results based on an index of affiliated patient organisation websites. Because the search results provided by Google are often not relevant or are of variable quality, EURORDIS has created a custom search engine to browse a specific selection of websites. Search results using this tool will provide results from the web pages of patient organisations who are members of EURORDIS as well as reference websites such as orpha.net and NIH.

Currently the service is only available in English.

EURORDIS Photo Contest 2013

.....

Initiated in 2006, the EURORDIS Photo Contest is an annual event that raises awareness about rare diseases to the general public, activates and builds our network of people living with a rare disease and their families and helps us collect photos which can be used in EURORDIS' printed materials, presentations and on the web.

The EURORDIS Photo Contest 2013 was open to public voting via social media for the first time.

The photo contest was a great success with 365 photos received from 47 countries representing more than 125 rare diseases. The launch of the photo contest through social media tripled EURORDIS' Facebook fans in the 3 months of the contest.

The winners were chosen by the EURORDIS Board of Directors and were announced via the EURORDIS eNews.

1



2



3



The Contest received hundreds of beautiful images. Thank you to all the participants who sent in a photo and to everybody who took the time to vote for their favourites!

- 1 The first prize went to Katerina from Greece, who lives with Friedreich ataxia. Katerina is a special needs teacher who lives a very active life.
- 2 The second-place winning photo depicts Lily and Tristan, twins living in France. They are the only known cases of mitochondrial cardiomyopathy with a mutation on the MRPL3 gene.
- 3 The third-place winning photo is of Margaretka, a little Slovakian girl living with dystrophic epidermolysis bullosa.

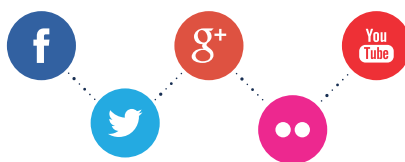
EURORDIS eNews

In April 2012, the EURORDIS newsletter evolved from a monthly to weekly format, allowing for the communication of breaking news of interest to the rare disease community more rapidly and frequently. Each weekly eNews features an in-depth, lead article devoted to important news in the Rare Disease community or to EURORDIS activity or events in addition to short headings of news which link to the relevant sections on eurordis.org.

In 2013 a more concerted effort was made to regularly streamline content from the eNews with communications made via social media tools, including Facebook and Twitter. The eNews lead story feeds the homepage on a weekly-basis in all 7 languages of the website (English, French, German, Italian, Portuguese, Russian and Spanish). An archive is also kept in the News and Events section of the EURORDIS.org website. A feature was developed on the website which links previous topic related articles to the lead story. The side-bar of the website provides a list of previous related eNews.



EURORDIS Social Media



EURORDIS has its own Facebook page (facebook.com/rarediseaseday), Twitter account (twitter.com/rarediseaseday), Flickr account (flickr.com/photos/eurordis), YouTube channel (youtube.com/eurordis), and Google+.

EURORDIS continued to leverage existing social media tools in order to communicate and interact with the rare disease community, and social media activity was robust in 2013.

In 2013, EURORDIS Facebook posts and updates received 474 000 views and generated 27 000 interactions with Facebook subscribers. The EURORDIS Facebook page received 12 500 "likes", reaching a total of 16 000 by the end of the year. EURORDIS Twitter was also active, with 337 tweets sent to over 4200 followers. EURORDIS uploaded 56 new videos on the EURORDIS YouTube channel. Altogether, these videos were viewed 17 000 times. In addition EURORDIS joined Google+ where it gained 67 followers.



Rare Disease Day 2013

.....

Rare Disease Day is an annual, awareness-raising event co-ordinated by EURORDIS at the international level and by National Alliances and Patient Organisations at the national level. The main objective of Rare Disease Day is to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives.

28 February 2013 marked the 6th edition of the Rare Disease Day Campaign with the slogan **"Rare Disorders without Borders"**. The campaign saw active participation in 72 countries, of which 36 European countries and 27 EU countries. EURORDIS and 29 Rare Disease National Alliances, together with patient groups acting as country organisers, mobilised thousands of patient organisations throughout 5 continents. There was participation for the first time in several countries including Bahrain, Iceland, Israel, Lebanon, Macedonia, Palestine, Singapore.

Around the world over 1000 unique events were staged on or around Rare Disease Day by patient organisations and other partners in order to raise awareness for rare diseases. EURORDIS organised a one-day Policy Event in Brussels entitled **"Faster Access to Medicines for Rare Disease Patients – The Transparency Directive"** (for further information refer to the "Rare Disease Day Policy Event" in the Advocacy section of this report).

The RDD 2013 website contained country pages, with a calendar of events organised in each country. The website included a downloadable tool kit with an Information Pack, logo, poster and other materials accessible to organisers; press releases for journalists and links to social media networks and a section for patients to upload photos and videos. Over January and February the website received over 75 000 unique visits with 18 000 visits on 28 February alone. Rare Disease Day 2013 received over 24 000 "likes" on Facebook and on Rare Disease Day itself there were 28,000 tweets, breaking down to 17 tweets per minute.

For the 2nd year in a row EURORDIS proceeded with the production of a Rare Disease Day video which was again a great success. EURORDIS worked directly with producer Carlo Hintermann to write and create this year's video with stop-motion technique, mixing animation and live participants. The video was available in 15 languages: English, French, Greek, Portuguese, Spanish, Italian, German, Hungarian, Catalan, Czech, Russian, Arabic, Hebrew, Mandarin, and Romanian. All creation and production costs were offered pro bono; EURORDIS covered only some direct technical expenses.

Hundreds of people responded to our call to Raise & Join Hands to show solidarity with rare disease patients on 28 February. Over 150 photos of people raising and joining hands were sent via the RDD website www.rdd.org and can be viewed on the RDD Flickr gallery.

Finally over 2013, a new website for Rare Disease Day was developed for 2014. The new website is more modern, including easier navigation for users visiting the site and is more adapted to scrolling with a tablet or smart phone. Links to social media are much more visible. The backend of the website has also been completely redone, facilitating organisations to upload pictures, videos, logos.



EURORDIS Policy Fact Sheets

EURORDIS has created specific Policy Fact Sheets to help rare disease patients and organisations better advocate issues of importance with national and/or European policy decision makers. The EURORDIS Policy Fact Sheets provide comprehensive, validated information on specific topics relevant to rare diseases. All these documents are available in printed form and online on eurordis.org in English and are widely distributed to members at relevant events.

In 2013, two new Policy Fact Sheets were elaborated and published: Adapted Housing Services, Resource Centres on Rare Diseases.



EURORDIS Black Pearl Gala Dinner

The second **EURORDIS Black Pearl Gala Dinner: Solidarity & Hope for Rare Disease Patients throughout Europe**, celebrating Rare Disease Day: "Rare Disorders without Borders" was held in Brussels, Belgium on 25 February 2013.

Over two hundred people attended the event – Brussels community members, national and international officials, researchers and other professionals striving to make treatments available to rare disease patients, individuals living with a rare disease, family and friends. They joined together to lift up the rare disease cause and recognise recipients of the EURORDIS Awards 2013.

The net proceeds of 282 k euros collected from the Gala support community building initiatives to break the isolation of rare disease patients and their families in Europe and empower leaders of the rare disease community through training, capacity-building activities and exchange, to stimulate research and increase public awareness about rare diseases.



EURORDIS Awards

.....

The EURORDIS Awards are designed to recognise the outstanding commitment and achievements of patients' advocacy groups, volunteers, scientists, companies, media and policy makers who have contributed – directly or indirectly – to reducing the impact of rare diseases on people's lives. These prestigious awards are judged

by the EURORDIS Board of Directors based on over 100 nominations received from EURORDIS members, volunteers and staff, with the aim of promoting leadership and the highest achievements in favour of people living with rare diseases.



2013 marked the second EURORDIS Awards for outstanding accomplishments in the field of rare diseases, which were presented in Brussels on the occasion of Rare Disease Day. The 2013 EURORDIS honorees were as follows:

| LIFETIME ACHIEVEMENT AWARD

Eva Luise Köhler, Former First Lady of Germany

In honour of her long record of dedication and commitment toward addressing the needs of people living with a rare disease.

| EUROPEAN RARE DISEASE LEADERSHIP AWARD

Ruxandra Draghia-Akli, MD, PhD

In recognition of her accomplishments as Director for Health Research at the European Commission and her leadership on behalf of the International Rare Diseases Research Consortium (IRDiRC).

| POLICY MAKER AWARD

MEP Françoise Grossetête

In recognition of her interventions in favour of legislations benefiting rare disease patients, in particular the Orphan Medicinal Products and the Paediatric Use of Medicines

| EURORDIS VOLUNTEER AWARD

Lesley Greene

In recognition of her outstanding and continuing dedication on behalf of the rare disease community

| SCIENTIFIC AWARD

Ségolène Aymé, MD

In recognition of overall scientific excellence and for the creation and development of Orphanet, the leading reference portal for expert validated rare disease and orphan drug information

| PATIENT ORGANISATION AWARD

Alström Syndrome UK

In recognition of their outstanding achievements, long-term commitment and participation in EU projects and initiatives

| COMPANY AWARDS

Celgene Corporation

In recognition of its established track record in the area of orphan diseases

Prosensa

In recognition of its innovation and promise and for its engagement with patient groups

Genzyme, a Sanofi Company

In recognition of the pioneering achievements and initiatives undertaken to ensure patient access to rare disease medicinal products

| MEDIA AWARD

Andrew Jack, Financial Times

In recognition of his contribution to improving understanding and awareness of issues relating to rare diseases.



HEALTH POLICY & HEALTH CARE SERVICES

Specialised Social Services and Social Policies

.....

In 2013 EURORDIS continued its focus on Specialised Social Services and on social policies, mainly through its involvement in Work Package 6 of the EUCERD Joint Action Working for Rare Diseases (EJA).

The definition of these Specialised Social Services continued to be disseminated via the concept paper entitled **'Rare Diseases: Addressing the Need for Specialised Social Services and Social Policies'** and via the distribution of Fact Sheets. Two new Fact Sheets were produced on Adapted Housing and on Resource Centres and the document on 'Guiding Principles for Specialised Social Services' was finalised and distributed to the EUCERD, to EURORDIS members and made available online via the EURORDIS and EUCERD websites.

Two workshops were organised at EMM 2013 Dubrovnik dedicated to the ten 'Guiding Principles for Specialised Social Services' and the presentation of case studies of expert services.

EURORDIS continued to map Specialised Social Services, as an ongoing exercise, in cooperation with National Alliances and European Federations, reaching over 70 services from 20 countries by the end of 2013. The map of these services, in addition to definitions, factsheets and testimonies of beneficiaries and volunteers can be found on the EURORDIS website in the designated section entitled **"Services to Patients: Specialised Social Services"**.

Country visits to BátorTábor Therapeutic Recreation

Services (April), and Group Homes for Prader-Willi Syndrome (October) were carried out in 2013 in order to collect extensive information on each of the services – using the script of 55 questions developed in 2012 – which will be used to develop case study documents.

In October, a Workshop on **'Training for Social Services Providers'** was organised at the House Of Disabled People's Organisations, Denmark, with 26 participants from 13 countries to reach consensus on baseline guiding principles, to be compiled in a document which will be distributed to EJA and EURORDIS partners in 2014.

In parallel to these actions, EURORDIS developed further tools to distribute all collected information among EURORDIS' members, patient advocates, policy makers, authorities and general public: a new section was created on the eNews and a new rubric was developed on Facebook in order to constantly distribute information on Specialised Social Services and social policies for people living with rare diseases. In parallel, EURORDIS maintained close connection with other partners (i.e. Orphanet) in order to spread information as well to the scientific/health professionals' communities (via Orphanews). By the end of 2013, over 70 000 people had been reached through these communication actions.

During 2013, EURORDIS connected to several partners working with social policies in order to exchange important information and good practices: Orphanet Disability Projects; Burqol-RD; International Federation of Social Workers.

RESEARCH, DRUGS & THERAPIES

EURORDIS intensified its direct role in advancing research in the field of rare diseases by expanding its efforts in strengthening ties with the research community to improve diagnostic and therapeutic possibilities for rare disease patients. More specifically, EURORDIS continued promoting the development of sustainable, harmonised

and integrated registry, biobanking and genetic data infrastructures and networks that reflect the patient's best interests. This was achieved by supporting the involvement of patients and their representatives at both the governance and clinical research levels of several international projects and consortia

Shaping and Supporting Research Policy

INTERNATIONAL RARE DISEASE RESEARCH CONSORTIUM – IRDiRC



The International Rare Disease Research Consortium (IRDiRC) teams up researchers and organisations investing in rare disease research in order to achieve two main objectives by the year 2020, namely to deliver 200 new therapies for rare diseases and means to diagnose most rare diseases.

EURORDIS strengthened its presence in the governing bodies of IRDiRC and participated in its meetings. Béatrice de Montleau, Board Member of EURORDIS, represented EURORDIS at the meetings of the Executive Committee of the Consortium, held in Barcelona, Spain, in January 2013 and in Dublin, Ireland, in April 2013; Valentina Bottarelli, EU Senior Policy Advisor, attended the September 2013 meeting, in Miami, USA.

Yann Le Cam, Chief Executive Officer of EURORDIS, was unanimously elected Chair of the Therapies Scientific Committee in October 2013, while Maria Mavris, EURORDIS' Therapeutic Development Director, continued to represent EURORDIS in the same Committee as well as in the Working Group on Orphan Drugs and Regulatory Processes. Anna Kole, EURORDIS Registry and Biobank Projects Manager, joined the IRDiRC Working Group on Registries and Natural History. Members of EURORDIS also joined working groups throughout 2013 including Tsveta Schyns (ENRAH and EURORDIS volunteer at EMA PDCO) became a member of the Working Group on Ethics and Governance, Nick Sireau (AKU) became a member of the Working Group on Chemically-derived products including repurposing, and Elisabeth Vroom (Duchenne Parent Project) became a member of the Working Group on Biotechnology-derived products including cell and gene based therapies.

In 2013, the Executive Committee of the IRDiRC adopted its "Governance Paper". EURORDIS contributed to the paper by submitting proposals aiming to ensure the presence

of rare disease patients in the governance of the IRDiRC and to improve the decision-making process by improving the dialogue between the Chairs of the three Scientific Committees, the Chair of the Executive Committee and the Scientific Secretariat of the Consortium.

The Therapies Committee, as well as the other two IRDiRC Scientific Committees, continued to work to lay out objectives, policies and guidelines of IRDiRC funded research projects. In particular, EURORDIS promoted a clearer and tighter agenda for the achievement of the overarching IRDiRC goal of having 200 more medicinal products for RDs on the market in 2020.

EURORDIS representatives attended the meetings of the respective governing bodies or participated in their conference calls. EURORDIS staff provided inputs and support to its representatives in IRDiRC, in particular by helping them prepare for their meetings and providing regular contribution to the key policy documents.

Finally, the first high level scientific conference of the Consortium was held in April 2013 in Dublin. A high level programme brought together scientists and other stakeholders in the field of RD research. EURORDIS speakers and panellists at the conference included Yann Le Cam, Maria Mavris, EURORDIS Vice President (also representing Fighting Blindness) Avril Daly, and Tsveta Schyns. Ahead of the Dublin Conference, EURORDIS issued a specific information sheet in support of patient representatives attending the event.

E-RARE

EURORDIS is a member of the External Advisory Board of E-Rare, the Era-Net project that has been established to develop synergies between eight public national research

programmes on rare diseases in the European Union, and to set up a coordinated research policy on rare diseases in Europe.



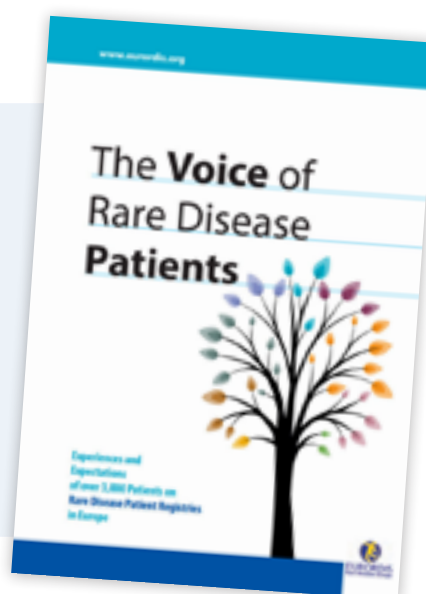
Promoting the Development of Effective Rare Disease Patient Registries

EPIRARE

The European Platform for Rare Disease Registries project (EPIRARE), was a three-year project co-funded by the European Commission's DG Health and Consumers within the EU Programme of Community Action in the field of Public Health. The aim of the project was to prepare the ground for the creation of an EU platform for the collection of data on rare disease patients. The Project addressed regulatory, ethical, technical and financial issues related to the development of rare disease patient registries.

In the context of the EPIRARE project EURORDIS also led the Work Package to develop policy scenarios on Registries and Data Collection engaging all concerned stakeholders in a consensual vision. These scenarios have progressively fed the Commission Joint Research Centre platform on Rare Disease Registries.

Within project EPIRARE, EURORDIS conducted an online survey to gather patients' thoughts and expectations on the topic of patient registries and the creation of a European Platform for Rare Disease Registries. The online survey was available in 11 languages: English (EN), French (FR), Italian (IT), German (DE), Spanish (ES), Portuguese (PT), Greek (EL), Romanian (RO), Czech (CS), Danish (DA) and Hungarian (HU). Over 3000 patients and parents affected by rare diseases across Europe participated in the survey whose results were compiled into a book and published by EURORDIS under the series, "**The Voice of Rare Disease Patients**".



TREAT-NMD

As a founding member of TREAT-NMD (Translational Research in Europe – Assessment and Treatment of Neuromuscular Diseases), EURORDIS continued its service to the newly governed "**TREAT-NMD Alliance**" as a member of the Executive Management Committee

and the Global Database Oversight Committee (TGDOC). Composed of academic and patient representatives, the Executive Management Committee governs the overall network whereas the TGDOC is responsible for reviewing all requests for data from the global database.



Promoting the Development of Sustainable and Integrated European Biobanks

.....

Patient organisations are aware of the need for more rapid progress in rare disease research and of the difficulties that researchers and clinicians face in obtaining human biological material to conduct research on rare diseases, and EURORDIS has represented this patient-driven view.

As the former coordinator of **EuroBiobank**, EURORDIS continued its strong commitment in promoting and actively participating in the governance of the first network of biobanks dedicated to rare diseases. EURORDIS continued to raise the importance of supporting biobanks for rare disease research to European and national level policy makers, liaising with the **Biobanking and Biomolecular**

Resources Research Infrastructure (BBMRI) Consortium, a 54-member consortium with more than 225 associated organisations (largely biobanks) from over 30 countries, which was officially awarded the Community legal framework for a European Research Infrastructure Consortium (ERIC) in November of 2013.

EURORDIS participated and presented at the annual 2013 BBMRI HandsOnBiobanks conference in October, advocating for closer connections between biobanks and patients or patient organisations as a vital element to advancing research.

Promoting the Sustainability, Integration and Harmonisation of Data Infrastructures

.....

Funded by the EU Seventh Research Framework Programme, RD-Connect will develop a global infrastructure for sharing outputs of research projects, starting with the EURenOmics project focusing on the causes, diagnostics, biomarkers, and disease models for rare kidney disorders, and the Neuromics project that will use next generation whole exome sequencing to increase genetic knowledge of rare neurodegenerative and neuromuscular disorders. The RD-Connect project supports the overarching IRDiRC 2020 goals.

EURORDIS joined as one of 27 full partners in RD-Connect, an integrated platform connecting databases, registries, biobanks, and clinical bioinformatics for rare disease research. EURORDIS is active in many facets of the project and thus has many opportunities to reflect the voice of patients throughout the various activities of the project. To structure the input of patient views in advising the governing board of the project, EURORDIS has established and continues to chair a 16-member Patient Advisory Council.

EURORDIS encouraged opportunities for patient involvement throughout each of the **RD-Connect** project work packages including the preparation and delivery of



educational materials and the consultation of RD patients on issues related to the research on “-omics” and the linkage of patient data across different infrastructures and across borders. Finally, EURORDIS identified opportunities for interaction and coordination of the RD-Connect network with other initiatives within and beyond Europe and the dissemination of the RD-Connect project outcomes at the international level to be carried out from this point forward.

A platform for sharing best practices for the management of rare diseases

.....

In January 2013, EURORDIS joined as one of 15 partners in the **RARE-Bestpractices project**, a 4 year (2013–2016) project Funded by DG Research under FP7-HEALTH-2012 INNOVATION-1. This project will develop a sustainable platform for sharing best practices for the management of rare diseases, supporting the collection of standardised and validated data and the efficient exchange of knowledge and reliable information on rare diseases. The expected impact of the project is an improvement of health outcomes and quality of life for rare disease patients, through the reduction of inequality in rare disease care and an access to better diagnostics and therapeutics at EU level. RARE-Bestpractices is aimed at collecting, evaluating and disseminating best practices and agreeing on a methodology suitable to develop and update best practice guidelines. The project will also create a forum for exchanging information, sharing lessons learnt and facilitating collaborations.

EURORDIS acts as a transversal partner in the different work packages, ensuring the involvement and the capacity-building of patient organisations across Europe regarding the project. EURORDIS is especially involved in the work packages focusing on dissemination, on

scientific coordination and networking with relevant activities in the field of rare diseases and on defining a common methodology for the production of guidelines on clinical management of rare diseases. In 2013, EURORDIS took an active role in the activities aimed at agreeing upon quality standards for the development of guidelines in the field of rare diseases, as well as in the development of communication tools. EURORDIS involved patient representatives in the project, organising workshops during the EURORDIS Membership Meeting in Dubrovnik and the meeting of the Council of European Federation. EURORDIS will continue to build capacities of rare disease patient representatives on the importance, use and benefits of best practices guidelines, and will involve them in the development of a pilot best practice guideline.



A platform for sharing best practices for the management of rare diseases

.....

EURORDIS continued its activities on Health Technology Assessment, focusing on acquiring a knowledge base, training rare disease patient representatives, gathering their experience in HTA procedures and promoting the involvement of patients in HTA procedures across Europe.

TECHNICAL AND SCIENTIFIC ACTIVITIES: THE EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT (EUNETHTA)

EURORDIS is one of the four patient representative organisations at the **EUnetHTA Stakeholders Forum** (together with the European Patient Forum, the European Multiple Sclerosis Platform, and the European Consumers Bureau BEUC). EUnetHTA is an EU initiative to improve coordination and harmonise the assessment tools used by the main HTA agencies in Europe. It is funded by the European Commission and Member States.

EUnetHTA is a joint action between the European Commission and Member States; it is now in the second joint action (JA2).

The current main objectives of EUnetHTA are to promote the actual use of methods and guidelines by HTA agencies at the national level and see which barriers may remain to better harmonise the assessment of the value of medicines across the EU, in order to decrease the disparity that exist regarding reimbursement of medicines in Europe.

EUnetHTA gathers 62 HTA agencies from the EU and beyond, and progress is presented regularly to the Stakeholders Forum. Members of the Stakeholders Forum represent not only patients and consumers, but also the pharmaceutical industry, payers (e.g. private insurance companies) and healthcare providers (e.g. hospitals).

 www.eunetha.eu

EURORDIS nominated its Director of Treatment Information and Access, Francois Houyez, to the position of co-chair of the Forum, and he was successfully elected.

POLITICAL AND STRATEGIC ACTIVITIES: THE EUROPEAN HTA NETWORK AT THE EUROPEAN COMMISSION

The Directive 2011/24/EU on the application of patients' rights in cross-border healthcare was adopted in 2011 and stipulates (Article 15) that the Union shall support and facilitate cooperation between national authorities or bodies responsible for health technology assessment designated by the Member States. According to the Implementing Decision, the HTA Network is to be supported by a scientific and technical cooperation to meet the objectives of the European cooperation on HTA as per Article 15 of the Directive.

The HTA Network held its first meeting on 16 October 2013 with representatives of all Member States and the European Commission. A delegation of five stakeholders' representatives met with the HTA Network, including EURORDIS. The HTA network is invited to define a long term vision for the provision of HTA in Europe, and a proposal should be developed in 2014.

Support to Patient Representatives at the European Medicines Agency

EURORDIS is in the unique position of having patient representation in the following European Medicines Agency (EMA) Committees and Working Parties: Committee for Orphan Medical Products (COMP); Paediatric Committee (PDCO); Committee for Advanced Therapies (CAT); Patients' and Consumers' Working Party (PCWP).

In addition to these permanent activities at EMA, patient representatives are also invited on a sporadic basis to attend the scientific committees and the Scientific Advice Working Party (SAWP) as experts for their disease.

In 2013, 16 patient representatives attended meetings of the SAWP for Protocol Assistance. Protocol Assistance is scientific advice for orphan medicinal products and is a way for the company developing the medicine to obtain scientific and regulatory advice on the manufacture, pre-clinical and clinical tests being performed. Patients are involved in order to provide first hand input on the most relevant outcome measures and endpoints for the clinical trials. The process of Scientific Advice/Protocol Assistance is recommended in order to avoid major objections regarding the design during evaluation of the marketing authorisation application.

The inclusion of patient representatives at the EMA either as permanent members of the committees and working party or sporadically is considered an important contribution by the Agency.

The Patients' and Consumers' Working Party, in which EURORDIS is a member and which is a unique forum where all scientific committees of the agency meet with patients and consumers, held four meetings in 2013. This working party organises the involvement of patients and consumers in all of the EMA activities (in 2012 a total of 525 patients/consumers were involved in one of the EMA activities). On the evaluation of benefit-risk for a marketing authorisation, even though patients are not yet members of the Committee for Human Medicinal Products (CHMP) at the EMA, the agency has put in place processes for the CHMP to consult with patients and their organisations when needed. One example is the consultation on a risk management plan for a new medicine to treat a rare cancer that could cause harm to the foetus during pregnancy. Measures on how to best minimise this risks were discussed and EURORDIS contributed.

Significant topics worked on in 2013 included: preparation for public hearings at the EMA (on medicines safety issues), shortages of medicines, communication on



The dedicated patient representative volunteers contributed this year to the examination of a total of 413 dossiers as part of the scientific committees they belong to. These include: 195 dossiers for orphan drug designations, 20 classification or certifications by the CAT and 198 Paediatric Investigation Plans by the PDCO.

The Therapeutic Action Group (TAG) composed exclusively of the EURORDIS representatives in the above-mentioned scientific committees and working party at the EMA continued their work and maintained communication internally with monthly conference calls and emails and had their annual meeting with the EURORDIS Board of Directors to exchange views on future actions.

additional monitoring of medicines, the ADVANCE project (studies on vaccines safety & effectiveness), the EMA's collaboration with HTAs (parallel scientific advice), a reflection on eligibility criteria for patients' and consumers' organisations to work with EMA, in particular financial aspects, the patient's voice in the evaluation of medicines

Lise Murphy, EURORDIS volunteer and member of Marfan Association Sweden, who was co-chair of the PCWP, stepped down in June 2013.

EURORDIS expressed its sincere gratitude to Lise Murphy, who spent valuable time as a volunteer to represent EURORDIS at the European Medicines Agency.

and involvement of patients in benefit/risk assessments at the EMA, the EU clinical trials register: presentation of results information, the update on the implementation of the new legislation on falsified medicines and the involvement of children and young people in the work of the paediatric committee.



THE DRUG INFORMATION, TRANSPARENCY AND ACCESS TASK FORCE (DITA)

The Drug Information, Transparency and Access Task Force (DITA) represents a group of volunteers who are trained (via the EURORDIS Summer School) and active in issues concerning therapeutic development of medicines for rare diseases. The Task Force supports and/or advises the EURORDIS representatives who participate in EMA Scientific Committees and Working Parties, or in the European Network of Health Technology Assessment (EUnetHTA) and the HTA Network (DG Sanco). It is consulted on papers prepared by EURORDIS.

DITA Task Force meetings were held in Dubrovnik in July 2013 (linked to EURORDIS Membership Meeting), and in London in December 2013.

The DITA Task Force membership was renewed in 2013 and is now composed of 18 members.

In 2013, DITA members exchanged information and elaborated contributions on medicines supply shortages (a common position between 22 patients', consumers' and healthcare professionals' organisations was adopted), contributed to the EMA public consultation on its new policy on clinical trial data transparency, reviewed 4 European Public Assessment Reports summaries for the Public, 14 package leaflets, 1 Art 20 safety referrals, 4 notes on supply shortages of medicines. Since 2007 when the procedure to review EPAR summaries and package leaflets (PL) was established for authorised medicines in the EU, 39 EPAR summaries and 56 PL were reviewed, for a total of 95 documents.

Among new actions, the DITA Task Force launched the **"Access Campaign"**: the Task Force is collecting evidence on access difficulties for medicines used in rare diseases in the context of the economic crisis, conducting literature searches, inviting national alliances and European federations to contribute.

In collaboration with the International Society of Pharmacoeconomics and Outcomes Research (ISPOR), EURORDIS, represented by Francois Houjé, is co-chairing the Patient Centred Special Interest Group.

DITA Task Force members participated in various conferences: EMA workshop on Medication Errors (28/02 – 01/03), with a presentation, EMA workshop of the European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA, 2 September), EMA workshop on Conflicts of Interests Policy (6 September), 7th EMA Stakeholder Meeting on the implementation of the new Pharmacovigilance legislation – 27 September, with a presentation, 4 meetings of the Patients' and Consumers' Working Party, and EMA training 10 December.



EURORDIS Capacity Building & Training for patient advocates

.....

In 2013, EURORDIS continued its capacity building & training activities for patient advocates through holding the highly successful EURORDIS Summer School for the 6th consecutive year; expanding its online e-learning tool; and continuing involvement in the DIA Patient Fellowship Programme. In 2013 collaboration continued with two training projects: the European Patients' Academy on Therapeutic Innovation (EUPATI) and the European Clinical Research Infrastructure Network – Integrating Activity (ECRIN-IA).

EURORDIS SUMMER SCHOOL

The EURORDIS Summer School was initiated in 2008 as part of our continued commitment to empowering people living with rare diseases. This four-day course provides training in aspects of medicines development and EU regulatory processes where patient representatives can be involved.

The 6th EURORDIS Summer School Session was held in Barcelona in June 2013 and once again aimed at training patient representatives in clinical trials, medicines development and EU regulatory processes. As in previous years, this four-day training gathered together a dynamic group. 2013 gathered 37 participants representing 15 countries and 39 different diseases.

A combination of small group sessions and formal presentations was used to introduce the concepts and terminology of clinical trials and to explain the roles of patient representatives at the European Medicines Agency both as committee members and as invited external experts involved in protocol assistance or communication activities for medicinal products.

For the second year in a row, non-rare disease patients were included as part of EURORDIS' collaboration with the European Clinical Research Infrastructure Network – Integrating Activity (**ECRIN-IA**) which develops e-services, education material to train professionals and patient associations, and communication with users, patients, citizens and policymakers (WP3). It supports the structuring and connection to ECRIN of disease-, technology-, or product-oriented investigation networks and hubs focusing on specific areas: rare diseases (WP4), medical devices (WP5), and nutrition (WP6). The inclusion of these non-rare disease patients was seamless and added an extra dimension for all participants involved.



ONLINE LEARNING

Based on the experience gained from the EURORDIS Summer School, a comprehensive e-learning tool covering topics such as clinical trial design, statistics and ethics has been designed and is freely available on the EURORDIS website. In addition, all presentations have been recorded, and downloadable pdf versions of the presentations are all available in the Training section of the website.

All of the above experiences will be further developed to include additional topics and to target different disease groups through the **European Patients' Academy on Therapeutic Innovation (EUPATI)**, a 5-year project funded by the Innovative Medicines Initiative (IMI). This patient-led academy will provide scientifically reliable, objective, comprehensive information to patients on pharmaceutical research and development.

The Consortium comprises a unique combination of pan-European patient organisations, academic and not-for-profit organisations as well as EFPIA member companies. EURORDIS is involved in the development of content, in particular for the areas of drug safety and risk/benefit assessment of (novel and existing) medicines, design and



objectives of clinical trials, and in the area of deployment where two face-to-face training sessions in 2015 and 2016, will be organised.

DRUG INFORMATION ASSOCIATION (DIA) PATIENT FELLOWSHIP PROGRAMME

The annual DIA Eurometing attracts over 3 000 representatives of the pharmaceutical industry, academia and public health authorities. Patient fellows are able to learn about regulatory affairs, clinical trials, latest drug developments, innovative therapies and meet with many different stakeholders. Since 2008 patient fellows have had a dedicated booth supported by EURORDIS in the exhibition hall, which has helped increase their visibility.

In 2013, the DIA Patient Fellowship Programme enabled 22 patient representatives from 15 different European countries to participate in the DIA 24th EuroMeeting in Amsterdam on 4-6 March 2013. In addition, 12 representatives of patient organisations were speakers in the EuroMeeting.



EURORDIS RoundTable of Companies (ERTC) – 2013 workshops

Initiated in 2004, the EURORDIS Round Table of Companies (ERTC) is a “club” of pharmaceutical companies with a common interest in rare diseases and orphan drug development.

In 2013, the ERTC comprises:

- 44 members
- 6 new members: bluebird bio, BMS/AstraZeneca, EUSA Pharma, Idis, Prosensa, Vertex Pharmaceuticals
- 2 upgrades: Actellon Pharmaceuticals Ltd. & PTC Therapeutics, Inc. both from the Sapphire to the Emerald level of ERTC membership



As is the case every year, two workshops were held in 2013:

“Corporate Responsibility in Improving Access to Orphan Medicinal Products” 27 February 2013, Brussels: 80 attendees from 15 countries

This workshop was focused on proposals aimed at achieving quick access to safe, efficient and affordable medicines with a real therapeutic added-value for rare disease patients in Europe. The importance of a continuum of data collection on medicinal products was emphasised, as well as early dialogue, differential pricing and National Plans. The Mechanism of Coordinated Access (MoCA) and Clinical Added Value of Orphan Medicinal Products (CAVOMP) are two examples of cross-country European initiatives. They aim at tackling bottlenecks by developing appropriate methodological tools and involving the right stakeholders, registries, development of a transparent value framework and national plans.

“Practical Aspects of Progressive Patient Access to Orphan Medicinal Products, Post-marketing Evidence Generation & Related Pricing Questions for Rare Disease Patients” 15 October 2013, Barcelona: 68 attendees from 12 countries

This workshop focused mainly on aspects of progressive patient access (or adaptive licensing), to improve access to orphan medicinal products (OMPs). The current approach for licensing of medicines needs to be revisited as therapies, for rare diseases in particular, cannot easily be evaluated by the “gold standard” – randomised clinical trials. Adaptive licensing would enable earlier access for patients to the medicinal product with a continuation of data collection and regulatory evaluation. Industry, regulatory and patient representatives were invited to voice their opinions and concerns for this proposal and the attendees contributed via the afternoon break-out sessions.

Compassionate Use Programmes for medicines

In June 2012, a request was made to the Heads of Medicines Agency to increase the transparency of compassionate use programmes (CUPs) authorised by their respective agencies. The proposal was made to inform the public of such programmes for example on the website of the heads of medicines agencies.

As a response, the Heads of Medicines Agencies (HMA) replied to EURORDIS on 25 July 2013 to inform us of a

decision of the HMA/EMA Taskforce on Transparency proposed to provide access to information on CUPs to patients in a single location linked to HMA and EMA. This was further discussed at the HMA Management Group which agreed that the national policies as regards to compassionate use programs in the Member States should be made more transparent and patients should be facilitated in finding relevant information.

GOVERNANCE, ORGANISATION & FUNDING

BOARD OF DIRECTORS

Four members were re-elected to the EURORDIS Board of Directors in 2013, following the elections at the Annual General Assembly in Dubrovnik: Christel Nourissier, representing the Alliance Maladies Rares; Terkel Andersen, representing the Danish Haemophilia Society; John Dart, representing DEBRA International, and Geske Wehr representing Selbsthilfelfchthyosee.V from Germany. The Board is composed of 12 members from 10 European countries.

In October 2013, Christel Nourissier resigned from the EURORDIS Board of Directors for personal reasons. She was replaced by Anne-Sophie Lapointe as the new representative of the Alliance Maladies Rares.

As of November 2013 the EURORDIS Board of Officers is composed of Terkel Anderson (President), Avril Daly (Vice-President), John Dart (General Secretary) Dimitrios Synodinos (Treasurer), Dorica Dan (Officer).



STAFF ORGANISATION

The team comprised 29 people, 26 full-time equivalent (FTE) as of December 2013. The team is composed of paid staff, one office volunteer, one consultant and two interns. Most staff members are based in the Paris office located in the Rare Disease Platform. EURORDIS' European Public Affairs Director and European Public Affairs Advisor are based in the Brussels office. The EURORDIS RareConnect team managing the online patient communities is based in Barcelona. The Chief Executive Officer shares his time between the Paris and Brussels offices.



The following are the main changes in human resources in 2013:

- Nina Miandabu, Accountant, has left EURORDIS
- Celine Parisse, Administrative Manager & Budget Controller, has joined EURORDIS
- Annie Rahazarizafy, Administrative & Support Services Assistant, has re-joined EURORDIS
- Minna Granger, Director of Outreach & Partnership and Communications & Development Unit Director, has left EURORDIS
- Mathieu Boudes, Operations and Projects Manager, has joined EURORDIS
- Monica Ensini, Scientific Director, has left EURORDIS
- Anna Kole, Registry and Biobank Projects Manager, has rejoined EURORDIS
- Juliette Senecat, Health Research Projects Manager, has joined EURORDIS
- Veronique George, Executive Assistant to the CEO, has left EURORDIS
- Tania Webster, Executive Assistant to the CEO, has joined EURORDIS
- Kasia Peala, Communications and Development Assistant, has left EURORDIS

Volunteers

.....

Most EURORDIS volunteers are either parents of patients affected with a rare disease or patients themselves. Due to the rarity of their disease and lack of available information, they have consequently become experts of their disease and of their respective national health care system. Other volunteers, indirectly affected by rare diseases, have also become very knowledgeable on rare disease related issues and are all very committed to the cause.

These volunteers are called the EURORDIS volunteer patient advocates.

They are driven by the urgent need to improve access to adequate diagnosis and care for rare disease patients, as well as access to adapted social services. Raising awareness is their credo.

EURORDIS is proud to rely on **54 dedicated volunteer patient advocates, one permanent office volunteer and 205 volunteer moderators** of Online Communities of Rare Disease Patients, within the activity "RareConnect".

The specific role of volunteer patient advocates consists in sharing their knowledge and experience to advocate for better national and European public health policy measures in favour of rare diseases. They participate in committees, working groups, Task Forces (DITA and RD Connect), and speak at international conferences.

Twelve volunteer patient advocates, parents of patients or patients, voice the rare disease patients' expectations in EU high-level committees of the European Medicines Agency and the European Commission.

OUR REPRESENTATIVES ON THE EMA SCIENTIFIC COMMITTEES

EMA Committee for Orphan Medicinal Products (COMP):

Ms Birthe Byskov Holm, Danish Osteogenesis Imperfecta Society and Rare Disorders, Denmark, full member
Ms Lesley Greene, EURORDIS, UK, Vice-Chair of the COMP
Dr Maria Mavris, Therapeutic Development Director, Observer (staff)

EMA Paediatric Committee (PDCO):

Dr Tsveta Schyns, European Network for Research on Alternating Hemiplegia, ENRAH, Belgium, full member
Dr. Gérard Nguyen, Rett Syndrome Europe, alternate

EMA Committee for Advanced Therapies (CAT):

Dr. Michele Lipucci di Paola, Associazione Veneta Lotta Talassemia, Italy, full member

Dr Monica Ensini, Scientific Director, alternate (staff)

EMA Patients' and Consumers' Working Party (PCWP):

Ms Lise Murphy, Marfan Syndrome, Sweden, PCWP Co-Chair

Mr Richard West, Behçets Syndrome Society, UK, full member

Mr François Houjiez, Information & Access to Therapies Director, member (staff)

OUR REPRESENTATIVES ON THE EU COMMITTEE OF EXPERTS ON RARE DISEASES (EUCERD) OF THE EUROPEAN COMMISSION

The eight seats (four full members and four alternates) for patients were held by EURORDIS representatives, all being patients or parents of patients. They represented both their respective patient organisations and EURORDIS. Yann Le Cam was the only staff member; other representatives were volunteers:

- Ms Dorica Dan, Romanian Prader Willi Association and Rare Diseases Romania
- Mr Jan Geissler, Leukämie-Online e.V, Germany
- Mr Alastair Kent, Genetic Alliance UK and Rare Disease UK
- Mr Yann Le Cam, EURORDIS
- Ms Christel Nourissier, EURORDIS and Prader Willi France
- Ms Bianca Pizzera, International Patient Organisation for Primary Immunodeficiencies, IPOPI, Italy
- Dr Gabor Pogany, William Syndrome and Rare Diseases Hungary

Flaminia Macchia, European Public Affairs Director, and Ariane Weinman, European Public Affairs Manager, were both observers on the EUCERD and supported the group of volunteers.

Torben Grønnebaek (Wilson Disease and Rare Disorders Denmark) was nominated to the EUCERD in 2010. He passed away in early 2012, leaving behind an invaluable heritage of courage. EURORDIS wishes to pay tribute to his outstanding contribution and dedication.

"I'D LIKE TO TAKE THIS OPPORTUNITY, ON BEHALF OF THE AGENCY, TO COMMEND ALL THE EURORDIS VOLUNTEERS WHO HAVE DEDICATED THEIR TIME AND EXPERTISE TO IMPROVE INFORMATION ON DRUGS FOR HUMAN USE. I WOULD BE GRATEFUL IF YOU COULD CONVEY OUR SINCEREST GRATITUDE TO THEM."

Dr. Isabelle Moulon, Head of Sector Medical Information of the European Medicines Agency (EMA)

Finance and Support Services

- Accounting and monthly financial reporting in a timely manner including cash flow and risk analysis detailed report.
- Monthly meetings with managers to update the Budget and the Year-end Financial Forecast.
- Operating Grant Steering Committees organised every two months to review progress on implementation, deliverables and budget.
- Quarterly collection of activity indicators.
- Management of human resources-related activities, such as recruitments and regulations
- Management of office support such as IT infrastructure, contact database, office supplies
- Management of legal and fiscal matters related to contractual matters

Contract Grants

NEW

RARE-Bestpractices – A platform for sharing best practices for the management of rare diseases, DG Health and Consumers, 48 months, 2013–2016

RD-Connect An integrated platform connecting registries, biobanks and clinical bioinformatics for rare disease research, associated partner, DG Research, 72 months 2012–18

RENEWED

Operating Grant for year 2013 ("EURORDIS_FY2013"), single beneficiary, DG Health and Consumers, 12 months

Epirare – European Platform for Rare Diseases Registries, associated beneficiary, DG Health and Consumers, 36 months, 2011–2014

ONGOING

Advocacy and core activities, AFM-Téléthon, 48 months 2010–2013

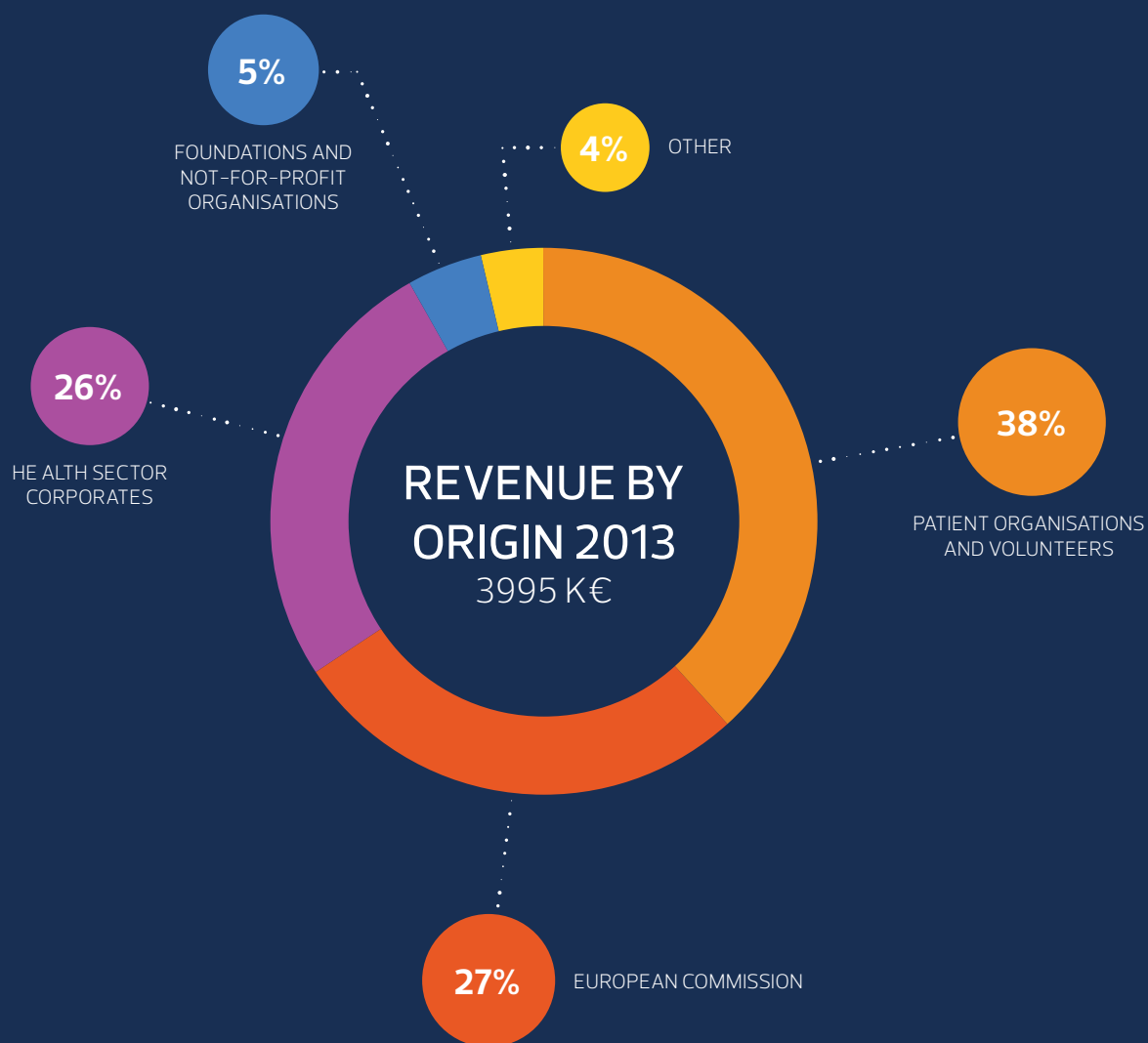
EJA EUCERD Joint Action, DG Sanco, 42 months, 2012–15

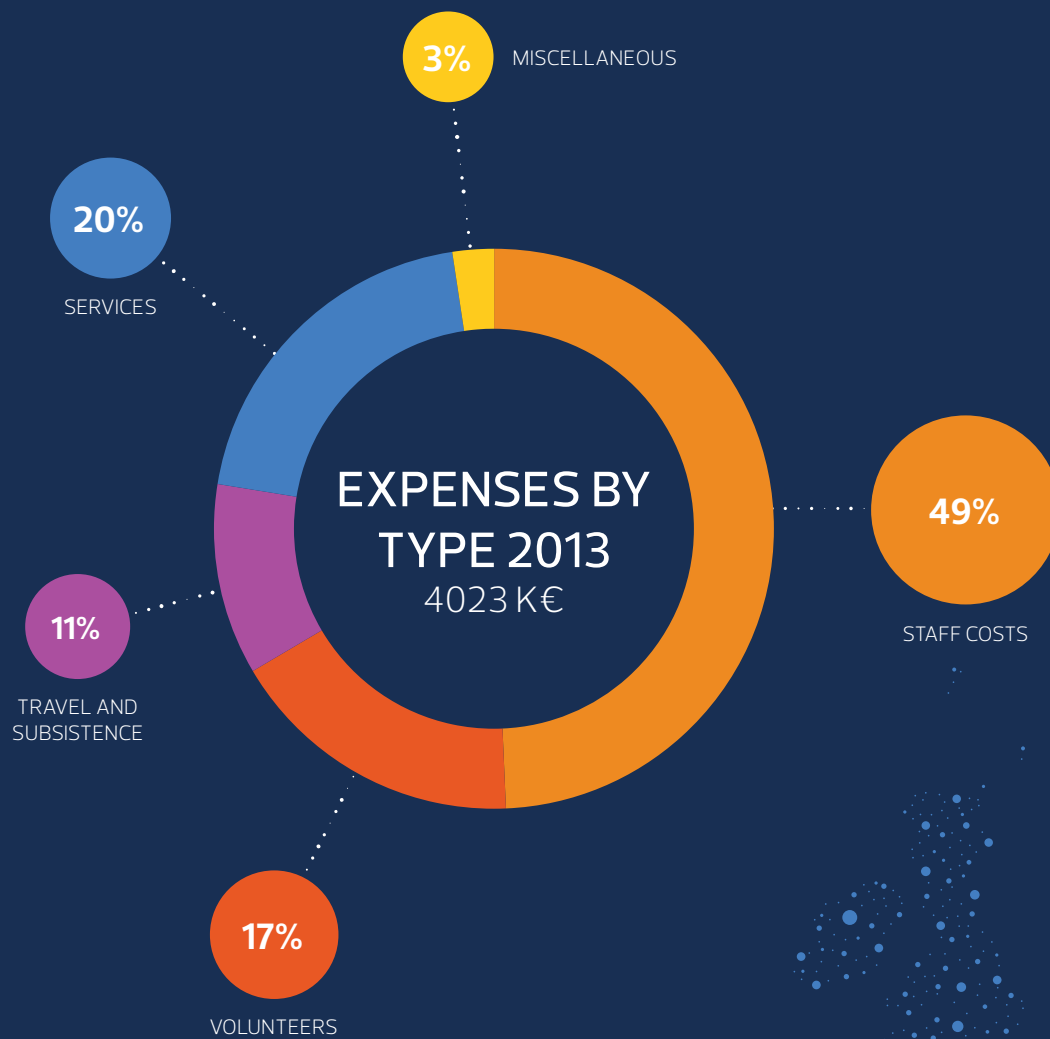
EUPATI European Patients' Academy on Therapeutic Innovation, Innovative Medicines Initiative, associated partners, – a DG Research / EFPIA Joint Undertaking, 60 months, 2012–16

ECRIN-IA European Clinical Research Infrastructures Network – Integrating Activity, associated partners, DG Research, 48 months, 2012–15



REVENUE & EXPENSES 2013







BOARD OF DIRECTORS MAY 2013 – MAY 2014

PRESIDENT		
Mr Terkel Andersen *President	Danish Haemophilia Society	Denmark
DIRECTORS		
Ms Simona Bellagambi	UNIAMO – Federazione Italiana Malattie Rare	Italy
Ms Avril Daly *Vice-President	Genetic & Rare Disorders Organisation	Ireland
Ms Dorica Dan *Officer	Romanian Prader Willi Association	Romania
Mr John Dart *Deputy General Secretary	DEBRA International	UK
Ms Birthe Byskov Holm	Rare Disorders Denmark	Denmark
Ms Béatrice de Montleau	AFM – Associations Française contre les Myopathies	France
Ms Christel Nourissier *General Secretary	Prader Willi France	France
Mr Dimitrios Synodinos *Treasurer	PESPA – Greek Alliance for Rare Diseases	Greece
Ms Rosa Sánchez De Vega	FEDER – Federación Española de Enfermedades Raras	Spain
Ms Geske Wehr	Selbsthilfe Ichthyose e.V.	Germany
Ms Vlasta Zmazek	Croatian Society for Rare Disorders	Croatia

In October 2013, Christel Nourissier resigned from the EURORDIS Board of Directors for personal reasons. She was replaced by Anne-Sophie Lapointe as the new representative of the Alliance Maladies Rares.

As of November 2013 the EURORDIS Board of Officers is composed of Terkel Anderson (President), Avril Daly (Vice-President), John Dart (General Secretary) Dimitrios Synodinos (Treasurer), Dorica Dan (Officer).

POLISH PKU AND RD ASSOCIATION «ARS VIVENDI»
POLISH SOCIETY OF MUCOPOLYSACCHARIDOSIS AND RELATED DISEASES
POLSKIE STOWARZYSZENIE NA RZECZ OSÓB Z AHC
THE DINA RADZIWIŁŁOWA CHILD'S HEART FOUNDATION

PORTUGAL

ALIANÇA PORTUGUESA DE ASSOCIAÇÕES DAS DOENÇAS RARAS
APLL – ASSOCIAÇÃO PORTUGUESA DE LEUCEMIAS E LINFOMAS
ASSOCIAÇÃO NACIONAL DE FIBROSE QUISTICA
ASSOCIAÇÃO PORTUGUESA CDG E OUTRAS DOENÇAS METABÓLICAS
ASSOCIAÇÃO PORTUGUESA DE DOENTES NEUROMUSCULARES
FEDRA – FEDERAÇÃO PORTUGUESA DE DOENÇAS RARAS
LIGUA NACIONAL PARA O ESTUDO E APOIO DA DEFICIÊNCIA MENTAL
NUCLEO DE EPIDERMOLISE BOLHOSA
RARISSIMAS – ASSOCIAÇÃO NACIONAL DE DEFICIÊNCIAS MENTAIS E RARAS

ROMANIA

ASOCIATIA COPILUL MEU-INIMA MEA
ASOCIATIA PERSOANELOR CU TALASEMIE MAJORA
ASOCIATIA ROMANA SPINA BIFIDA SI HIDROCEFALIE
ASOCIATIA WERDNIG HOFFMAN AWH
ASOCIATIA WILLIAMS SYNDROME
ROMANIAN MYASTHENIA GRAVIS ASSOCIATION
ROMANIAN NATIONAL ALLIANCE FOR RARE DISEASES
ROMANIAN PRADER WILLI ASSOCIATION

RUSSIAN FEDERATION

ASSOCIATION FOR ASSISTANCE TO RETT SYNDROME PATIENTS
HELP TO CYSTIC FIBROSIS PATIENTS
HUNTER SYNDROME ASSOCIATION
INTERREGIONAL PUBLIC ORGANISATION FOR GAUCHER DISEASE
NATIONAL ASSOCIATION OF PATIENTS WITH RARE DISEASES «GENETICA»
NGO «FRAGILE CHILDREN»
RUSSIAN INTERREGIONAL PUBLIC ORGANIZATION «INTER-REGIONAL SUPPORT CENTRE FOR PATIENTS WITH ANIRIDIA «IRIS»
RUSSIAN PATIENT UNION – RARE DISEASES WORKING GROUP
THE ASSOCIATION OF PRIMARY IMMUNODEFICIENCY PATIENTS

SERBIA

CHILD RARE DISEASE SUPPORT AND RESEARCH ASSOCIATION LIFE
CHILDHOOD CANCER PARENT ORGANISATION «ZVONICA»
NATIONAL ORGANIZATION FOR RARE DISEASES, SERBIA

SINGAPORE

RARE DISORDERS SOCIETY (SINGAPORE)

SLOVAKIA

DEBRA SR
ORGANISATION OF MUSCULAR DYSTROPHY IN THE SLOVAK REPUBLIC
SLOVAK ALLIANCE OF RARE DISEASES
SLOVAK CYSTIC FIBROSIS ASSOCIATION
ZDRUZENIE OJEDLINELYCH GENETICKYCH OCHORENIE

SLOVENIA

DEBRA SLOVENIA – DRUSTVO DEBRA SLOVENIJA
EAMDA EUROPEAN ALLIANCE OF NEUROMUSCULAR DISORDERS ASSOCIATIONS
FABRY PATIENTS ASSOCIATION SLOVENIA / DRUŠTVO BOLNIKOVA S FABRYJEVO BOLEZNIJO SLOVENIJE
FOUNDATION OF CHILD NEUROLOGY

SOUTH AFRICA

PRIMARY IMMUNODEFICIENCY NETWORK OF SOUTH AFRICA
--

SPAIN

ALIANZA ESPAÑOLA DE FAMILIAS DE VON HIPPEL LINDAU
ALPHA EUROPE FEDERATION
ASOCIACIÓ CATALANA DE LAS NEUROFIBROMATOSIS
ASOCIACIÓ D'AFECTATS DE SÍRINGOMELIA
ASOCIACIÓN ANDALUZA CONTRA LA FIBROSIS QUISTICA
ASOCIACIÓN ANDALUZA DE PACIENTES CON SÍNDROME DE TOURETTE Y TRASTORNOS ASOCIADOS

ASOCIACIÓN BALEAR DE AFECTADOS POR LA TRIGONITIS Y LA CISTITIS INTERSTICIAL
ASOCIACION CHIARI Y SÍRINGOMELIA DEL PRINCIPADO DE ASTURIAS
ASOCIACIÓN DE AFECTADOS DE NEUROFIBROMATOSIS
ASOCIACIÓN DE AFECTADOS POR DISPLASIA ECTODÉRMICA
ASOCIACIÓN DE DEFICIENCIAS DE CRECIMIENTO Y DESARROLLO
ASOCIACIÓN DE EPIDERMOLISIS BULLOSA DE ESPAÑA (DEBRA ESPAÑA)
ASOCIACIÓN DE ESCLERODERMIA CASTELLON
ASOCIACION DE FAMILIARES Y AFECTADOS DE LIPODISTROFIAS
ASOCIACION DE FAMILIARES Y ENFERMOS DE FPI Y FPF
ASOCIACIÓN DE HEMOGLOBINURIA PAROXÍSTICA NOCTURNA
ASOCIACIÓN DE HUESOS DE CRISTAL DE ESPAÑA
ASOCIACIÓN DE LAS MUCOPOLISACARIDOSIS Y SÍNDROMES RELACIONADOS
ASOCIACIÓN DE NEVUS GIGANTE CONGÉNITO
ASOCIACIÓN DEL SÍNDROME DE LESCH-NYHAN ESPAÑA
ASOCIACION ESPAÑOLA DEL SÍNDROME CDG, DEFECTOS CONGENITOS DE LA GLICOSILACION
ASOCIACIÓN ESPAÑOLA DE ANGIOEDEMA FAMILIAR POR DEFICIT DE C1
ASOCIACIÓN ESPAÑOLA DE ANIRIDIA
ASOCIACIÓN ESPAÑOLA DE ENFERMOS DE GLUCOGENOSIS
ASOCIACIÓN ESPAÑOLA DE ENFERMOS Y FAMILIARES DE LA ENFERMEDAD DE GAUCHER ESPAÑA
ASOCIACIÓN ESPAÑOLA DE ESCLERODERMIA
ASOCIACIÓN ESPAÑOLA DE FAMILIARES Y ENFERMOS DE WILSON
ASOCIACION ESPAÑOLA DE FIBRODISPLASIA OSÍFICANTE PROGRESIVA
ASOCIACIÓN ESPAÑOLA DE ICTIOSIS
ASOCIACIÓN ESPAÑOLA DE PARAPARESIA ESPÁSTICA FAMILIAR STRÜMPPELL-LORRAIN
ASOCIACIÓN ESPAÑOLA DE PORFIRIA
ASOCIACIÓN ESPAÑOLA DEL SÍNDROME DE JOUBERT
ASOCIACIÓN ESPAÑOLA SÍNDROME DE SJÖGREN
ASOCIACIÓN HHT ESPAÑA
ASOCIACIÓN NACIONAL AMIGOS DE ARNOLD CHIARI
ASOCIACIÓN NACIONAL DE AFECTADOS POR SÍNDROMES DE EHLERS DANLOS E HIPERLAXITUD
ASOCIACIÓN NACIONAL DE HIPERTENSIÓN PULMONAR
ASOCIACIÓN NACIONAL SÍNDROME DE APERT Y OTRAS CRANEOSINOSTOSIS SINDRÓMICAS
ASOCIACIÓN SÍNDROME ANGELMAN
ASOCIACIÓN SÍNDROME DE LOWE DE ESPAÑA
ASOCIACION STOP SANFILIPPO
ASSOCIACIÓ CATALANA DE SÍNDROME DE SJÖGREN
ASSOCIACIÓ DE LLUITA CONTRA LA DISTONIA A CATALUNYA
ASSOCIACIÓN CATALANA DE ENFERMEDADES NEUROMUSCULARES
D'GENES ASOCIACIÓN DE ENFERMEDADES RARAS DE MURCIA
DRAVET SYNDROME FOUNDATION, DELEGACION EN ESPAÑA
EUROPEAN NETWORK FOR RARE AND CONGENITAL ANAEMIAS
FEDERACIÓ CATALANA DE MALALTIES POC FREQUENTS
FEDERACIÓN ANDALUZA DE ASOCIACIONES DE ATAXIAS
FEDERACIÓN DE ATAXIAS DE ESPAÑA
FEDERACIÓN ESPAÑOLA DE ENFERMEDADES NEUROMUSCULARES
FEDERACIÓN ESPAÑOLA DE ENFERMEDADES RARAS
FEDERACIÓN ESPAÑOLA DE FIBROSIS QUISTICA
FEDERACIÓN ESPAÑOLA DE HEMOFILIA
FEDERACION ESPANOLA DE PADRES CON NIÑOS CON CANCER
FEDERACIÓN ESPAÑOLA DEL SÍNDROME X FRÁGIL
FUNDACION MENUDOS CORAZONES
FUNDACIÓN NIEMANN PICK DE ESPAÑA
FUNDACIÓN SÍNDROME DE MOEBIUS
FUNDACION SÍNDROME DE WEST
MIQUEL VALLS FOUNDATION (FUNDACION PRIVADA CATALANA DE ESCLEROSI LATERAL AMIOTROFICA)

SWEDEN

AGRENSKA
EHLERS-DANLOS SYNDROM RIKSFÖRBUND SVERIGE
EUROPEAN DYSMELIA REFERENCE INFORMATION CENTRE



CONFERENCES & WORKSHOPS 2013

Financial Times Global Pharmaceutical and Biotechnology Conference, London, UK, 4 December

Yann Le Cam, panellist in the session 'Europe in crisis – Managing for Uncertainty'

INFRAFRONTIER meeting, Rome, Italy, 2–4 December

Maria Mavris represented EURORDIS

Colloque handicaps rares –Parole croisée parent – professionnel: “Les parcours de vie des personnes en situation de handicap rare – Discontinuités – transitions – ruptures”, Paris, France, 2–3 December

Raquel Castro and Ariane Weinman represented EURORDIS.

RARE 2013: « L'innovation et les partenariats au service des maladies », Montpellier, France, 28–30 November

Christel Nourissier: Atelier « Relations associations de malades et industrie : suivi de l'atelier de RARE 2011 »

International GSD (glycogen storage disease) Conference 2013, Heidelberg, Germany, 28–30 November

Geske Wehr: “Living with a rare disease”

Workshop on “HTA EMA Parallel Scientific Advice”, London, UK, 26 November

Yann Le Cam: “Why is this needed? Where do we want to get to? The Patient Advocate View”

RD Connect: “HandsOn Biobanking”, the Hague, Netherlands, November 21–22

Anna Kole: “Patient input in biobank and patient registries”

« L'accompagnement, un nouveau droit des malades ? » (social care, a new right for patients ?) Paris, France November 20

Raquel Castro represented EURORDIS.

World Orphan Drug Congress 2013, Geneva, Switzerland, 15 November

Yann Le Cam: “Improving Global Access to Orphan Medicinal Products: Gathering the Concepts & Innovative Policy Proposals in a Comprehensive Approach”

“Scientific Support for Public Health: Existing actions, new challenges and European Added Value”, European Commission, Brussels, Belgium, 14 November

Yann Le Cam: “RARE DISEASES: Challenges for science based policy making”

Flaminia Macchia represented EURORDIS as well

“Meet the Cystic Fibrosis Patients & Care Providers: How can Europe help to improve health outcomes and survival for children and young adults with Cystic Fibrosis”, European Parliament, Brussels, Belgium, 14 November

Juliette Sénécat represented EURORDIS

CORD Conference: "Consultations Toward A Canadian Plan for Rare Diseases", Toronto, Canada, 12 November

Yann Le Cam: "Policy initiatives in Europe to improve access to OMPs and promote a sustainable business model"

"Cross-Border Healthcare in Europe: Towards a Patient-Centred System", Brussels, Belgium, 12 November

Flaminia Macchia represented EURORDIS

ISPOR 16th Annual European Congress, Workshop: Dublin, Ireland 2-6 November

François Houÿez: "Launch of the Patient Centres Special Interest Group"

Empatient Worskhop: « Emergence de compétences dans le domaine de l'accès à l'innovation thérapeutique » (capacity building in the field of access to therapeutic innovation). Paris, France 23 October

Nancy Hamilton: "European Patients' Academy on Therapeutic Innovation"

2nd International EPIRARE Workshop "Rare Diseases and Orphan Drug Registries, Rome, Italy, 21-22 October

Monica Ensini, Chair of session

15th TIF International Thalassaemia Conference for Patients and Parents, Abu Dhabi, 20-22 October

François Houÿez: "Common Position of Patients and Healthcare Professionals on Medicines Supply Shortages"

AIRG Belgium (Organisation for Information and Research on Genetic renal diseases), Charleroi, Belgium 20 October,

Yann Le Cam: « Les Maladies Rares en Europe et en Belgique »

200th ENMC (European Neuro-Muscular Centre) International Worskhop, NH hotel in Naarden, the Netherlands, 18 October

Flaminia Macchia: Speech on the concept of European Reference Networks

RD Connect: Stakeholders Conference on Ethical Legal Social Issues, Brussels, Belgium, October 17-18

Anna Kole: "Patient Perspectives on Data Sharing"

Orphan Drug and Rare Disease Seminar, Marseille, France, 17-18, October

Maria Mavris: "Patients' Organisation in Orphan Drug Development"

EHC Round Table: Haemophilia Centres of Expertise in Europe, European Parliament, Brussels, Belgium 17 October

Flaminia Macchia: "European Reference Networks: The patient perspective"

Juliette Sénécat represented EURORDIS as well.

10th TOPRA Annual Symposium 2013, Lisbon, Portugal, 16 October

Session on the Clinical Trial Regulation Proposals

Flaminia Macchia: "EURORDIS- A patients' Group viewpoint on the Revision of the Clinical Trials Legislation"

1st Ibero-American Meeting of Rare Diseases Associations Representatives, Totana, Spain, 14-20 October

Yann Le Cam: "What is the state of the art regarding advocacy for People Living With Rare Diseases in Europe?"

John Dart: "European Reference Networks: The patient perspective"

Anja Helm: "Rare Disease Day"

European Medicines Agency, Workshop on Medicines Supply Shortages, London, UK, 14 October

François Houÿez: "Common Position of Patients and Healthcare Professionals on Medicines Supply Shortages"

Pharmaceutical Users Software Exchange, "Patient Centricity", Brussels, Belgium, 14 October

François Houÿez: Keynote speech: "Patients Acting Against their Own Disease"

RARE Best practices Workshop, Freiburg, Germany, 11-12 October

Juliette Sénécat and **Geske Wehr** represented EURORDIS

NORD-DIA US Conference on Rare Diseases and Orphan Products: "The New Era in Health Care", North Bethesda, USA, 7-9 October

Yann Le Cam, panellist in the session on the International Perspective on Orphan Drugs / Devices

Towards a European Reference Network for Genordermatoses, Istanbul, Turkey, 5 October

John Dart: "The Key Role of Patient Association"

Geske Wehr also represented EURORDIS

VI Foresight Training Course "Biotech and Innovative Science to meet Patient Needs", (The Gianni Benzi Foundation), Bari, Italy 30 September-2 October

François Houÿez: "Medicines Supply Shortages"

CORD conference, Ottawa, Canada, 30 September

Yann Le Cam: "European Rationale and Plans - EU Guidelines"

European Medicines Agency, 7th Stakeholders' Meeting on Pharmacovigilance, London, UK, 27 September

François Houÿez: "How French media covered recent safety referrals"

European Medicines Agency, Patients/Consumers Working Party (PCWP) and Healthcare Professionals Working Party (HCPWP) joint meeting, London, UK, 26 September

François Houÿez: "Common Position of Patients and Healthcare Professionals on Medicines Supply Shortages"

Lunch Debate on Data Protection at the European Parliament, hosted by Marielle Gallo (MEP) and organised by EURORDIS & EPIRARE— Brussels, Belgium, 26 September

John Dart, Monica Ensini, François Houÿez, Yann Le Cam and Flaminia Macchia represented EURORDIS.

1st Congresso Iberoamericano de Doenças Raras, Brasilia, Brazil, 24–25 September

Yann Le Cam: "EURORDIS and the Power of Patient Organisations"

XIVth Forum of Associations, Orphanet, New IT for the collection and sharing of data on rare diseases, Paris, France, 23 September

François Houÿez: "Transparency of Clinical Trials Data and Results"

IIV Escuela de Formación CREER -- FEDER 2013, Burgos, Spain, 20–21 September

Marta Campabadal: "Presentación del servicio FEDER– EURORDIS para crear comunidades online de ámbito europeo: la Red Social RareConnect"

1st World Conference on Congenital Disorders of Glycosylation for Families and Professionals: "a booming story of sugar trees", Barcelona, Spain, 31 August– 1 September

Rob Pleticha: Presentation of RareConnect

"A Roadmap for Sharing Clinical Trial Data", Brussels, Belgium, 27 August

Flaminia Macchia represented EURORDIS

9th EFIM Clinical Research Seminar, Paris, France (The Foundation for the Development of Internal Medicine in Europe And The European Federation of Internal Medicine), 10 July

François Houÿez: Round Table: "protection of persons in clinical trials"

Workshop on "EU Data Reforms and What They Mean for Research", Wellcome Trust and European Foundations Centre, Brussels, Belgium, 2 July

Jill Bonjean represented EURORDIS

Audition publique – « Médecine personnalisée : enjeux éthiques et sociétaux », Assemblée nationale, Paris, France, 25 June

Yann Le Cam – Table Ronde: "Quels regards des patients et des associations de malades"

TOPRA, the Organisation for Professionals in Regulatory Affairs, Brussels, Belgium, 18 June

François Houÿez: "Patients and Pharmaco-vigilant!"

« L'Innovation Thérapeutique » Séance commune de l'Académie des sciences, de l'Académie nationale de Pharmacie et de l'Académie des technologies, sous le haut patronage du Ministère de l'Enseignement Supérieur et de la Recherche et du Ministère des Affaires Sociales et de la Santé, Paris, France, 11 June

Yann Le Cam: « Participation des Associations de Malades à l'évaluation des Médicaments. Conséquences positives »

European Medicines Agency, Patients/Consumers Working Party (PCWP) and Healthcare Professionals Working Party (HCPWP) joint meeting, London, UK, 5 June

François Houÿez: "Patients and Healthcare Professionals views on supply shortages of medicines: Discussion of a common position"

Rett Syndrome conference, Nantes, France, 18 May

Christel Nourissier: "Presentation of EURORDIS"

International Meeting of Porphyria Patients, Lucerne, Switzerland, 17 and 18 May

Anja Helm: "Rare Together: Creation, Operation and Management of European Federations"

Rob Pleticha: "RareConnect"

2013 NORD Corporate Council Meeting, Washington DC, 14 May

Jill Bonjean represented EURORDIS

"European Brain Research: Successes & Next Challenges", organised by the European Commission – Brussels, Belgium, 14 May

Monica Ensini represented EURORDIS

IMI (Innovative Medicines Initiatives) Stakeholder Forum 2013: "Public-private partnerships for health research and innovation", Brussels, Belgium, 13 May

Monica Ensini represented EURORDIS

European Multiple Sclerosis Platform Spring Conference: "Better together – sharing expertise and influence to improve access to treatment, care and employment", Brussels, Belgium, 2–4 May

Maria Mavris: "Building, supporting and equipping advocates for effective lobbying"

Second Balkan Patients Meeting: "Better Communication – Better Treatment", Sofia, Bulgaria, 20–21 April

Vlasta Zmazek: "EUCERD Recommendation on Quality Criteria for Centres of Expertise in Member States"

1st Conference of the International Rare Diseases Research Consortium (IRDiRC), Dublin, Ireland, 16–17 April

Yann Le Cam: "Regulatory Dialogue to Optimise Orphan Drug Development: Patients' Experience and Perspectives"

Maria Mavris: Challenges: "Challenges: the Patients' perspective"

Avril Daly: "Patient-Led Initiative to Identify the Molecular Cause of Rare Inherited Retinopathies"

Béatrice de Montleau represented EURORDIS in the IRDiRC Executive Committee.

Annual Conference on European Pharmaceutical Law, organised by ERA (Academy of European Law (ERA), Brussels, Belgium, 11–12 April

François Houÿez represented EURORDIS

| 3rd Annual World Orphan Drug Congress, Washington D.C., USA, 9–11 April

Yann Le Cam, Chair of the first day: "What European patients expect from sponsors and regulators: a patient perspective on lessons and opportunities for improvement"

| EB–CLINET Register Workshop, Vienna, Austria, 4–5 April

Monica Ensini: "EURORDIS/EPIRARE Patient Survey on Registries"

| European Waldenström's Macroglobulinemia network Affiliates' Meeting, London, UK, 16 March

Marta Campabadal: "Presentation of RareConnect"

| Seventh European Workshop "Brains for Brain", Frankfurt, Germany, 8–10 March

Monica Ensini: "Research on Rare Diseases: International and patient inclusive"

| 25th Annual DIA EuroMeeting, Amsterdam, The Netherlands, 4–6 March

Lise Murphy: "Patient View on Risk Communication"

Yann Le Cam: "Early access approaches to medicinal products for rare disease patients: features and perspectives"

Maria Mavris, Session Chair, Meeting with patient DIA fellows and students DIA fellows

| CORD –Canadian Organization for Rare Disorders – 5th Rare Disease Day Conference: "From Worst to First: Transforming the Rare Disease Experience in Canada", Toronto, Canada, 28 February

Yann Le Cam: "European Approach in Improving Access to Orphan Medicinal Products: Concepts & Proposals Toward a Comprehensive Approach"

| Workshop on "Medication Errors", European Medicines Agency, London, UK, 28 February

François Houjé: "Medication errors: what patients can do to minimise them"

| Patients/Consumers Working Party (PCWP) and Healthcare Professionals Working Group (HCP WG) joint meeting, London, UK, 27–28 February

Maria Mavris: "Preparing patients' representatives for Protocol Assistance"

| 3rd Rare Disease Day Meeting organised by ABAIMAR (the balearian association for rare diseases), Palma de Mallorca, Spain, 27 February

Marta Campabadal: "Presentation of RareConnect"

| 2° Congresso Regionale "Malattie Rare: La Vita E' Ancora Belloa...Diagnosi, Terapia, Qualita di Vita", Bari, Italy, 21–23 February

Simona Bellagambi: "Malattie rare: una rete di competenze, cervelli e cuori" (Rare Diseases: a network of competences, brains and hearts)

"La Rete transfrontaliera dei Centri di Competenza: indicazioni per i centri regionali"

| Workshop "Advanced Therapy Medicinal Products", European Parliament Brussels, 20 February

Monica Ensini: "The Voice of Patients"

| Atypical Haemolytic–Uremic Syndrome: 1st Meeting of European aHUS Patient Groups, Barcelona, Spain, 16 February

Marta Campabadal: Presentation of RareConnect

| ADEDIEM Association des anciens Etudiants du DESS et du Master Développement et Enregistrement International des Médicaments de l'université Paris XI, Châtenay–Malabry, France, 4 February

François Houjé: "Pharmaco–vigilant patients and transparent regulators"

| Consultation on the Future of European Public Health Research – organised by the European Commission, Brussels, Belgium, 30 – 31 January

Christel Nourissier represented EURORDIS

| EFGCP Annual Conference 2013 & 20th Anniversary: "Virtual Future: the ethical dimensions of emerging technologies in clinical trials and research", Brussels, Belgium, 29 – 30 January

Denis Costello represented EURORDIS

| "The Future of Clinical Trials" – European Parliament Brussels, Belgium, 28 January

Flaminia Macchia participated as EURORDIS representative

| "Health Outcomes in Europe: the QALY controversy", European Commission DG Research, Brussels, Belgium, 25 January

François Houjé: "QALY–based appraisals and rare diseases"

| 4th E–Rare Workshop: "Ethical Aspects of Exome and Whole Genome Sequencing Studies in Rare Diseases" Tel Aviv, Israel, 14 January

Monica Ensini participated as EURORDIS representative

ACKNOWLEDGEMENTS

EURORDIS would like to thank the following organisations and companies for their financial support in 2013:

Not-for-Profit Organisations and Public Entities

• • • • •

AFM – Téléthon

The "Association Française contre les Myopathies" (AFM – Téléthon), for the annual core activities grant and the office space they make available to the organisation free of charge.



CNSA

« Caisse Nationale de Solidarité pour l'Autonomie » (CNSA) for co-funding of the EUCERD Joint Action Working for Rare Diseases (EJA), WP6 « Specialised social services »



EUROPEAN COMMISSION

European Commission (DG Health and Consumers) for its essential contribution to the following activities:

- The Operating Grant for year 2013 (EURORDIS_FY2013)
- The European Platform for Rare Diseases Registries (EpiRare)
- The EUCERD Joint Action Working for Rare Diseases (EJA)



Co-funded by
the Health Programme
of the European Union

European Commission (DG Research and Innovation) for its essential contribution to the following projects:

- RD-Connect Project to improve connections among different clinical datasets such as registries, biobanks and clinical bioinformatics for rare disease research.
- The European Clinical Research Infrastructures Network Integrated Activity (ECRIN-IA) project, which partners with and supports the EURORDIS Summer School
- The Innovative Medicines Initiative-Joint Understanding (IMI-JU) project: European Patients' Academy on Therapeutic Innovation (EUPATI)
- RARE-Bestpractices – A platform for sharing best practices for the management of rare diseases



**AIPM, Association
of International
Pharmaceutical
Manufacturers**

For co-funding the Russian version
of EURORDIS Website and eNews



EFPIA

For co-funding the Innovative
Medicines Initiative–Joint
Understanding (IMI–JU) project:
European Patients' Academy on
Therapeutic Innovation (EUPATI)



**Fondation du LEEM
– Les entreprises du
médicament**

For co-funding of RareConnect
Online Patients Communities



Medtronic Foundation

For co-funding of the EJA EUCERD
Joint Action, DG Sanco, WP4
Support for the implementation
of plans or strategies at MS level
(continuation of EUROPLAN)



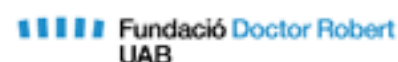
**Fonds Léa Rose, sous
l'égide de la fondation
Roi Baudouin**

For co-funding of the EJA EUCERD
Joint Action, DG Sanco, WP6
Specialised social services and
integration of RD into social policies
and services



**Fundació Doctor Robert
Universitat Autònoma
de Barcelona Casa
Convalescència**

For the office space they make
available to the RareConnect staff
free of charge.



RarePartners

For EURORDIS Black Pearl Gala
Dinner contribution



Pharmaceutical and Biotechnology Sector Companies

.....

EURORDIS believes that diversification of funding is a key success factor to minimise potential conflict of interest with donors. In 2013, 40 Companies have supported EURORDIS through the EURORDIS Round Table of Companies¹, the EURORDIS Membership Meeting 2013 Dubrovnik, the RareConnect Online Patient Communities², the EpiRare project and the EURORDIS Gala Dinner³ which funds actions to reduce isolation of people living with rare diseases, to increase awareness of rare diseases and to empower leaders of rare disease communities through training, capacity-building and exchange. The breakdown of each company's donations by project is detailed on the EURORDIS website on the "Corporate" tab of the "Financial Information" section.

THE TOP FIVE DONORS

CELGENE



GENZYME



GSK



SHIRE HGT



SIGMA TAU



¹ <http://www.eurordis.org/content/ertc-members>

² <http://www.rareconnect.org>

³ <http://galadinner.eurordis.org>

OTHER PHARMACEUTICAL COMPANIES (LISTED ALPHABETICALLY)



OTHER HEALTH SECTOR CORPORATES

Idis For EURORDIS Black Pearl Gala Dinner 2013 contribution



Marc Krueger & Associates, Inc. For EURORDIS Black Pearl Gala Dinner 2013 contribution and support to the ERTC Workshop Welcome Dinner

Mark
Krueger & Associates, Inc.

OTHER CONTRIBUTORS

Burson-Marsteller For on-going in-kind contribution of communications advice to build awareness of rare diseases



Multiburo For the discounted pricing of the office space made available to the Public Affairs team based in Brussels



Lottomatica For EURORDIS Black Pearl Gala Dinner 2013 contribution



Ares For EURORDIS Black Pearl Gala Dinner contribution



WORKPLAN²⁰¹⁴



EURORDIS
Rare Diseases Europe

ACTION PLAN 2014	64
BUDGET 2014	76
GOVERNANCE CHART 2014	78
TEAM CHART 2014	80





OUR ACTION PLAN FOR 2014

Advocacy

.....

ADVOCACY ISSUES

- Turning rare diseases into a sustainable public health priority in the European Union
- Making rare diseases a public health priority in all EU Member States
- Promoting rare diseases as a public health priority internationally
- Improving access to orphan drugs and treatments for rare diseases
- Improving quality and access to rare disease diagnosis and care
- Accessing cross-border healthcare and making possible patient mobility
- Promoting research and bridging patients and researchers
- Addressing the issues of genetic testing, genetic counselling & newborn screening

ADVOCACY ACTIONS

- **Support implementation of the EU policy framework adopted in 2008 “Commission Communication on Rare Diseases: Europe’s Challenges” and in 2009 “Council Recommendation on an Action in the Field of Rare Diseases” through:**

- 1 Participation in the European Commission Expert Group on Rare Diseases:

Participation of four EURORDIS' patient representatives and their four alternates in the Commission Expert Group on Rare Diseases – CERD (replacing the EU Committee of Experts on Rare Diseases –EUCERD)

- 2 Participation in the European Commission Expert Group on Cancers – CEC:

Apply for participation of Rare Cancer patient organisation representatives and their alternates in the European Commission Expert Group on Cancers

- 3 The EURORDIS Policy Action Group (PAG):

- The patient representatives in the Commission Expert Groups together with public affairs staff form the EURORDIS Policy Action Group (PAG).
- The PAG teams up with other volunteers and EURORDIS staff on the issues of the two Commission Expert Groups:
 - National Strategies / National Plans & Indicators
 - Centres of Expertise and European Reference Networks
 - Cross-border Healthcare
 - Registries and Data Collection
 - Codification
 - Guidelines on Diagnostics and Care

- Gene Testing and Counselling
 - Information Provision to Patients and Professionals
 - Access to Rare Disease Therapies and Orphan Drugs
 - Specialised Social Services
 - Integration of Rare Diseases into Social Policies and Services
 - The PAG contributes to the Commission Expert Groups with concrete policy proposals in order to address patient needs when implementing the strategy foreseen in the EU policy framework
 - The PAG intends to lead and support the Commission Expert Group on Rare Diseases collaboration in the following specific areas: Rare Disease Day, European Conference on Rare Diseases and Orphan Products, Information Provision to Patients and Professionals, Improvement of Access to Medicines, Comprehensive approach to address the Health and Social needs of People Living with Rare Diseases (PLWRD), and the promotion of international cooperation
 - Contribute actively to the "State of the Art of Rare Disease Activities in Europe" through direct EURORDIS contributions and involvement of its National Alliances
- 4 EUCERD Joint Action Working for Rare Diseases (March 2012–September 2015):
- EURORDIS supports the implementation of the EUCERD Joint Action as a full partner across all Work Packages and disseminates its outcomes (EURORDIS is the NGO representing patients in this Joint Action involving the EU Member States and the European Commission).
 - EURORDIS is specifically involved in two Work Packages of the EUCERD Joint Action:
 - Leader of Work Package 6 on "specialised social services and integration of rare diseases into social policies and services"
 - Co-partner of Work Package 4 on the "support for the implementation of plans or strategies for RDs at MS level" (continuation of EUROPLAN)
 - Engage EURORDIS in the planning of the 2nd Joint Action Working for Rare Diseases
 - Engage EURORDIS in the planning of the 1st Joint Action for Rare Cancers
- **Disseminate EU policy outcomes, monitor progress, promote new strategies and innovative solutions, to integrate all main stakeholders at EU and national levels, and share common objectives through ECRD 2014 Berlin, internal dissemination, EURORDIS eNews and EURORDIS Website**
- **Maintain rare diseases as a sustainable policy and budget priority in the EU policy programmes for the period 2014– 2020:**
- As a public health priority in the 3rd EU Public Health Programme "Health for Growth" & Annual Work Programmes and advocate on EURORDIS priorities
 - As a research priority in the 8th EU Research & Technology Framework Programme "Horizon 2020" & biannual Work Programmes and in the Innovative Medicines Initiatives (IMI 2), and advocate on EURORDIS priorities
- **Monitor the implementation of the EU Directive on Patient's Rights to Cross-border Healthcare (Patients' Mobility) adopted in 2011 providing a legal basis for patients' rights for cross-border care and for the establishment of European Reference Networks aimed at addressing specific needs of rare disease patients and families.**
- Monitor the real life experience of patients on the national transposition
 - Gather and exchange experience of National Alliances
- **Advocate to improve access to orphan drugs in the context of the financial and economic crisis both to prevent or limit impact of cut back measures and promote a new business model sustainable for society**
- Promote and take an active part in relevant dialogue on Medicine Alternative Development Pathways
 - Support and monitor implementation of the Clinical Added Value of Orphan Medicinal Products (CAVOMP) process based on the EUCERD Recommendation, in conjunction with EMA, EUnetHTA, MEDEV, the EuropaBio–EBE Taskforce on Rare Diseases and Orphan Drugs
 - Promote innovative concepts, policies and pilots: Early dialogue / Scoping / De-Risking, EMA-HTA–Payers dialogue involving patient representatives and medical experts, Progressive Patients' Access / Adaptive Licencing, Compassionate Use and Early Access Programmes, Shortages, and Off-label Use of Medicines in Rare Indications
 - Contribute to the new approaches to Benefit/Risk evaluation, taking into consideration the patient values & patients' preferred treatment options, in order to improve the patient information and to improve well informed medical decisions
 - Contribute to the pilots of the Mechanism of Coordinated Access (MOCA) to Orphan Drugs, developed within the EU Stakeholders Forum on Corporate Responsibility in Pharmaceuticals, promoting pan-European collaboration between EU Member States on Orphan Drugs based on a common approach to the Value of new medicines, pricing, volume and post-Marketing Authorisation data generation
 - Explore the wider use of Managed Entry Agreement as well as Differential Pricing
 - Contribute to adaptive approaches on methodology and criteria for the Health Technology Assessment of the Effectiveness or Relative Effectiveness as well as for evaluation of the value of Orphan Drugs, for potential use at a European level

- Promote these innovative approaches in Member States & National Plans on Rare Diseases
- Increase direct cooperation with leaders from RD Therapies sector
- In emergency situations, support actions to defend patients' access to rare disease therapies

| Organise a European Policy Discussion Event in Brussels for Rare Disease Day 2014 on 25 February 2014 on "Improving Access to Rare Disease Care: The Vision of Patients"

| Promote rare diseases as an international public health priority through:

- The launch and development of "Rare Diseases International" as an informal network of rare disease patient organisations with the possible organisation of Rare Diseases International meeting back to back with IRDiRC conferences 2014–2015
- The international spreading of Rare Disease Day
- The adoption of a Joint Declaration "Rare Diseases: an International Public Health Challenge" by major rare disease umbrella patient organisations
- Exploring ways of collaboration between Rare Diseases International and ICORD
- Elaboration of a strategy toward the UN System: ECOSOC and WHO, and Council of Europe

| Advocate in support of rare disease research: A high priority in the Strategy 2010–2015

- Contribute to the consolidation of RD priorities and activities in Horizon 2020, IMI 2, JRC, ERA-NET, eRare, IRDiRC
- Engage EURORDIS in the European research infrastructures, policy debates and projects
- Promote IRDiRC objectives, governance and contribute to IRDiRC policy papers
- Empower patient advocates through Policy Factsheets and capacity building workshops at the EMM 2014 Berlin

| Advocate on the impact of new knowledge on genetics through the:

- Elaboration of EURORDIS positions on genetic testing and screening
- Development of a knowledge base and shaping future EU legislation on anti-genetic discrimination
- Contribution to the reflection processes on personalised medicines

| Prepare for the EU pharmaceutical legislation revision

Information & Networking

.....

COMMUNITY BUILDING

| Maintain the EURORDIS Membership over 600 members and ensure regular interaction

| Start implementation of new process of regular membership reassessment of eligibility criteria

| Recruitment of members at large, particularly in all EU member states, acceding and candidate EU Member States, rare cancer groups and European Federations

| Organise EURORDIS Membership Meeting 2014 Berlin on 8 May with Capacity Building Workshops

| Examine the feasibility of building on the Summer School to expand capacity building of rare disease patient advocates in Social Media & Communications; Social Policy; Research

| Build capacities of the European network of 33 National Alliances through:

- Promoting greater convergence and collaboration between National Alliances, as well as between National Alliances and EURORDIS, through the shared process "Common Goals & Mutual Commitments between National Alliances in Europe and EURORDIS: An agenda between 2014 & 2020".

- Sharing information, experience, guidance and common actions in National Plans, Rare Disease Day, Cross-border Healthcare, Access to Medicines and Evaluation of Centres of Expertise
- Organising one Workshop of the Council of National Alliances
- Organising telephone or web based conference calls on specific topics
- Maintaining direct interaction or visits between EURORDIS and National Alliances
- Operating a EURORDIS "Learning from Each Other" Exchange Programme for National Alliances to enable more direct exchange, mutual support and capacity building between National Alliances
- Encouraging contact between patient organisations in same countries and the creation of new national rare disease alliances where there are none yet

| Build capacities of the European network of 41 disease specific European / International Federations & Networks through:

- Sharing information, experience, good practices and guidance – integration of RareTogether! Website in Council of European Federations (CEF) section – between European Federations and common actions in European Reference Networks, Rare Disease Day, drug development & interaction with EMA & access

to medicines & patients reporting of adverse events, cross-border healthcare, social policy & services, and Online Patient Communities

- Providing regular advice to European Federations & Networks
- Organising a two day workshop of the Council of European Federations, including a one day training
- Expanding the EURORDIS Programme to Support European Federations & Networks with seed money for their governance meetings, membership meetings, first European conferences on their disease
- Operating a EURORDIS "Learning from Each Other" Mutual Exchange Programme for European Federations or Networks to enable more direct exchange, mutual support and capacity building between disease specific networks or federations

■ Outreach to patient groups in Central & Eastern Europe, Balkans, Russia and Caucasia and support their actions to raise public awareness, promote policy on rare diseases and promote the creation of national alliances:

- Maintain and expand EURORDIS Website, eNews and main documents available in Russian
- Provide official EURORDIS endorsement, promote and take part in national conferences across Central & Eastern Europe on request of our members
- Support Russian and Caucasian endeavours in raising awareness of rare diseases in their regions through support of their national conferences or EURORDIS-EUROPLAN conferences on national RD strategies as well as Rare Disease Day.
- Provide access to EURORDIS Patient Advocate Fellowships Programmes for the EURORDIS Membership Meeting 2014 Berlin and to the EURORDIS Summer School 2014 Barcelona
- Maintain dissemination of Decide Topics available in 22 languages

■ Support EURORDIS Volunteers involvement through:

- Strengthening the identification, recruitment and support of volunteers to be increasingly involved in EURORDIS activities, participating in NGO partnerships and representing patients in European Commission and EMA working groups and committees
- Briefing on key topics, access to shared reference documents and public presentations
- Creating a volunteer section on the EURORDIS website
- Adopting a global Charter of EURORDIS Volunteers emphasising the core values of EURORDIS

■ Expand "RareConnect", the EURORDIS-NORD Social Networks of Online Patients Communities in five to seven languages:

- Adopt a RareConnect Action Plan for RareConnect for 2014-2016 and a Strategy for 2020

- Adjust Governance and Management structure to new development phase
- Maintain & support the 50 existing Online Patient Communities in conjunction with the over 370 patient groups involved and 183 volunteer moderators
- Launch 20 new Online Patient Communities, adapting the tool to each specific community needs in conjunction with relevant existing patient groups for these rare diseases
- Develop an evolved translation model for RareConnect based on developing a plan seeking investment in a volunteer translator management tool along with a volunteer translator programme including outreach to translation institutions, sourcing volunteer translators from other sources and the incorporation of part-time or volunteer coordinator into the team.
- Add an additional two languages to RareConnect (e.g. Russian and Portuguese)
- Enhance the presentation of information on existing communities to bring conversations and patient testimonials to the fore and improve the presentation and quality of Medical Information.
- Develop the possibility for RareConnect to create Topic Groups. Topic Groups will be lighter to setup and maintain than full blown communities and be purely conversation driven. They will be moderated however and depending on the experiences or topics, moderators can include EURORDIS Staff, Expert Patients or Members of Learned Societies or Networks. Launch approximately 10 Topic Groups in 2014. Possible Topics can include: "Integration at school", "Diagnosis & Gene Testing", "Epileptic Seizures", "Nutrition & Growth", "Reimbursement of Care and Treatments", "Cross-border Healthcare", "Clinical Trials", "Registries & Biobanks", "Good Practices on Diagnostics & Care", "Social & Financial Impacts of Rare Diseases".
- Pilot RareConnect as a platform to perform surveys
- Invest in a mobile interface for RareConnect to enable visitors to participate on a more regular basis and accomplish priority tasks from their mobile device
- Develop partnerships with National Alliances, but also where appropriate and where there is crossover (with topic groups for example) with Learned Societies
- Redefine partnership with NORD in terms of their involvement in the project

INFORMING & RAISING AWARENESS

■ Organise Rare Disease Day 2014: on the theme/slogan "Join together for Better Care" keeping the overarching theme of "Solidarity" until 2016

- Launch a new dedicated, user-friendly Rare Disease Day website with more features and information, including Rare Disease Day events being organised around the globe
- A promotional video in over 20 languages to be disseminated through a social media campaign and the EURORDIS websites

- Marketing & Communication Plan: Info Pack, Poster & Slogan, patient testimonies, social media
- Expand to 80+ countries in Europe and at the international level
- Continue to expand the Join and Raise Hands campaign and tie it in to social media campaign
- Organise RDD at midnight around the world: a new global collaborative effort at midnight (in different time zones) on 28 February
- Storify: A digest of social media and Country pages information disseminated in the weeks leading up to 28 February

Organise a Policy Discussion Event in Brussels on the occasion of Rare Disease Day 2014 on 25 February 2014 on "Improving Access to Rare Disease Care: The Vision of Patients"
Plan Rare Disease Day 2015:

Marketing and Communication Plan –Info Pack, video, Poster & Slogan, Website, patient testimonies, social media, Rare Disease Day Event in Brussels

- RDD 2015: Plan Rare Disease Day Event in Brussels

Organise the EURORDIS Awards 2014 designed to recognise the outstanding commitment and achievements of patients' organisations, volunteers, companies, scientists, media and policy makers who have contributed to reducing the impact of rare diseases on people's lives with:

- Nominations open to the public for the first time
- Announcement of shortlisted nominees prior to the Awards
- Dedicated website section on eurordis.org

Take actions towards a European Year for Rare Diseases (EYRD) in 2019:

- Adopt communication plan for the European Year for Rare Diseases campaign
- Launch of the EYRD campaign in 2014 building the base of ambassadors and patrons
- Launch of a social media campaign and identity to reach out to patient organisations, people living with rare diseases, policy makers and stakeholders

Maintain content quality and information architecture of EURORDIS Website:

- Centered on target audiences: patient organisations & advocates, patient and families. Other stakeholders (secondary)
- Maintain navigation and user friendliness of website
- Maintain quality, updated information in seven languages (EN, FR, DE, ES, IT, PT, RU) in all sections
- Further develop content and facilitate access via three focal points: the Core EURORDIS.org website, its Satellites – (eNews, Rare Disease Day, RareConnect, Help Lines) and the EURORDIS Social media – (Facebook, Twitter, Flickr, YouTube, Rare Disease Blogs)

- Within core website consolidate issues of strategic importance, adding dedicated sections on Compassionate Use, Volunteers, and the European Year of Rare Diseases and updating sections on What We Do, Rare Disease EU Policy, News & Events
- Promote the EURORDIS Rare Disease Blogs and involve National Alliances, European Federations, volunteers and key actors
- Better organise the publications section so that important documents such as Q&A, Fact Sheets are more visible and easily accessible
- Implement the Social Media Strategy and Internal organisation for more vibrant Social Media such as the Blog, Facebook, Twitter, Flickr, YouTube
- Develop strategy for promotion of EURORDIS website's main new services, sections, information, and capacity-building tools for patients and patient advocates

Improve access to patients' generated knowledge through Social Media:

Continue Social Media and Patient Empowerment Webinar Series to moderators and patient groups

Maintain EURORDIS TV which provides updated video footage of:

News & Current Affairs, Living with Rare Diseases, Patient Groups, Research, Health Policy, Orphan Drugs & Therapies, EURORDIS speeches & events, conference presentations, training or capacity building videos, Rare Disease Day events through the EURORDIS website:

- Make use of pre-existing recorded material, edit and upload to EURORDIS TV
- Record videos – in house and outsourced – to present key policy issues / papers / Summer School and EMM testimonies to also be provided on EURORDIS TV
- Rebrand EURORDIS TV and further promote it

Publish EURORDIS eNews in 7 languages (EN, FR, DE, SP, IT, PT, RU) on a weekly basis all year round (except August), 45–48 issues

Present Photo Contest 2013 at EMM 2014 Berlin

Organise Photo Contest 2014 with an eventual presentation at EMM 2015 Madrid

Conceive a deeper and long-term strategy on photo and video projects to promote people living with rare diseases in the public space

INFORMATION SERVICES TO PATIENTS

Improve access to and quality of information through Rare Disease Help Lines:

- Disseminate Policy Fact Sheets and guideline on the creation & development of national help lines and the

European Network of Help Lines

- Develop the European Network of Rare Diseases Help Lines:
 - Governance & business meeting, develop membership base
 - Explore new financial tools for the help lines (Structural Funds)
 - Submit and publish an article on the Caller Profile Analysis in a scientific journal
 - Maintain shared tools, e.g., common software for the collection of data on enquiries, develop new functions and update disease classification, and organise the annual caller profile analysis
 - Organise local trainings on shared tools and searching quality information on the web

- Engage and train help lines on reporting adverse events of medicines used in all rare disease treatments (whether orphan or not, and off-label) as part of the EURORDIS DIOD project (Daily Impact of Drugs in patients' lives) or of other activities
- Develop a strategy towards obtaining an EU wide unique 116 number
- Link to National Plans
- Link to ERNs

■ **Maintain and improve the recently launched EURORDIS InfoHub feature on the eurordis.org website and RareConnect Platform to improve access to quality information sources on the internet for patients, families and relatives as well as structured access and use of information for patient advocates**

Health Policy & Healthcare Services

.....

PROMOTING RARE DISEASE HEALTH POLICY DEVELOPMENT

■ Organise the 7th European Conference on Rare Diseases and Orphan Products— ECRD 2014 Berlin on May 8–10th 2014:

- Prepare the Programme and the Programme Committee:
 - Coordinate the multiple partnerships with DIA, NORD, CORD, ACHSE, Commission Expert Group on Rare Diseases, EMA, Orphanet, ESHG, EFIM, EBE/EFPIA–EuropaBio.
 - Create Programme Committee (Co-chairs: Lesley Greene, Wills Hughes–Wilson and Ségolène Aymé) and Develop Programme. 3. Promote ECRD & registrations in six languages (EN, FR, ES, DE, RU, PO). Plan Call for Posters, renew EURORDIS Patient Advocates Fellowship Programme, organise logistics of sessions selected to be simultaneously translated, develop communication around the event, outreach to medical journals to encourage higher attendance from healthcare professionals
- Plan satellite workshops for partners eg. Orphanet, IRDiRC ExCom

■ Support the National Alliances for their action in advancing national rare disease policy through the following activities:

■ EUCERD Joint Action / Work Package 4: “support for the implementation of plans or strategies for RDs at MS level” (continuation of EUROPLAN):

- Support 20 National Alliances in the European Union to facilitate the organisation of a EUROPLAN National Conference aimed at promoting the implementation of a national plan for Rare Diseases.

- Support 4 to 5 National Alliances (or Patient Groups) in European countries outside the EU to facilitate the organisation of a EUROPLAN National Conference.
- The EUROPLAN National Conferences use the same format and methodological tools derived from EUROPLAN I (2008–2011). However the organisers can adapt the layout of the Conferences to the most pressing needs of the RD community in their countries.
- 10 EURORDIS Advisors, all from different National Alliances, are in charge of advising one to three National Alliance(s) for the preparation of the EUROPLAN National Conference. The 10 EURORDIS Advisors team up with the EURORDIS public affairs staff.
- The 10 EURORDIS Advisors participate in the EUROPLAN capacity building workshops together with representatives of national authorities.
- Work on common policy scenarios for small EU countries (less than 5 million)

■ EURORDIS Membership Meeting 2014 in May in Berlin

- Information & experience sharing across members of EURORDIS on national planning
- Capacity building workshop for rare disease patient advocates: promoting national plans, proposing adequate measures, playing an active role in national plan steering committees, being trained in new policy areas.

■ EURORDIS Policy Fact Sheets for Patient Advocates

- 2–page document enabling patient representatives to better advocate issues of specific importance with national and/or EU policy decision makers (e.g. on policy and services expected by patients and their families)

- Development of new Policy Fact Sheets on: Access to Orphan Medical Products; OrphaCode and its national potential use; EU structural funds for National Plans; Update of European Reference Networks; Classification of functioning for PLWRDs

| Targeted communication

- Promote exchange of information and sharing of experiences through the website by expanding the more user-friendly EURORDIS web sections on EU and national policies, the eNews and the participation in national conferences or other meetings.
- Develop new ways to exchange information, experience, good measures, and concerns between National Alliances, the 10 EURORDIS Advisors and the Staff – such as “national plan mailing list” and the eNews.

PUTTING RARE DISEASE PATIENTS AT THE HEART OF THE HEALTHCARE SYSTEM

| Promote policy on European Reference Networks & Centres of Expertise & Expert Networks & Healthcare Pathways on rare diseases:

- Promote the long term EURORDIS strategic vision on European Reference Networks on Rare Diseases addressing patient needs
- Disseminate the Declaration of Common Principles on Centres of Expertise & European Reference Networks
- Disseminate the Patients' Preferred Policy Scenario on centres of expertise
- Disseminate “Good Practices of Collaboration between Patient Organisations in Centres of Expertise and European Reference Networks”, based on POLKA
- Develop future possible transversal actions to support the collaboration between European Federations, patients organisations or communities and European Reference Network (ERNs), based on the new EU policy of ERNs in rare diseases, in key areas such as governance of ERNs, disease registries, biological repositories, clinical trials, treatment protocol trials, standards of diagnosis & care, standards of social & supportive care, information to patients, outreach to patients
- Organise a theme in ECRD 2014 Berlin on “Improving Healthcare Services” which includes European Reference Networks and Centres of Expertise
- Maintain a dedicated Website section on Centres of Expertise and European Reference Networks

| Participate in the development of new long-term projects on the methodologies for creation and review of best clinical practices:

- Partnering in the EU project RARE-Bestpractices, “Platform for sharing best practices for management of rare diseases”, in order to:

- 1 build capacities of rare disease patient organisations and of people living with rare diseases on the importance, use and benefits of best practice guidelines, through training activities targeted at patient advocates and dissemination of information and project outcomes (WP1)
 - 2 establish a EURORDIS working group on best clinical practices
 - 3 contribute to the development and the implementation of a core methodology on best practice guidelines for rare diseases (WP3)
 - 4 ensure a strong interaction and coordination of the RARE-Bestpractices project with other initiatives such as EUROPLAN II, RD-Connect and IRDIRC and contribute to the dissemination of the RARE-Bestpractices project outcomes (WP1,6).
- How to better use scientific progress in biomedical research to translate results into tangible benefits for RD patients and to offer high quality information to patients and health-care professionals;
 - How to promote and support a consistent level of healthcare services for RD patients in the EU while implementing the EU Directive on Patients' Rights in Cross-border Healthcare.

| Promote policy on newborn screening, gene testing, pre-implantation diagnostics:

Create a dedicated website section and disseminate information, including better promotion of information available from EuroGentest, European Society of Human Genetics and International Society for Neo-Natal Screening.

| Contribute to the transparency of Clinical Trials data (both global results and individual patient's data):

Adopt a position on the EMA policy on access to clinical trials data

INTEGRATING RARE DISEASES INTO SOCIAL POLICY AND SPECIALISED SERVICES TO PATIENTS

| Promote integration of the challenges faced by people living with rare diseases into social policy:

- Within the EUCERD Joint Action (EJA) (2012–2015), EURORDIS leads the Work Package on “Provision of Specialised Social Services and Integration of Rare Diseases into Social Policies and Services”: mapping existing Specialised Social Services and collecting guiding principles for these services; developing case studies on experienced services based on information collected during country visits; addressing issues related to the training of Specialised Social Services providers
- Also within the EJA, EURORDIS will coordinate with the work package on National Plans, ensuring the integration of the different issues concerning social challenges into the content outline – minimum requirements and recommended content for the workshops of EUROPLAN National Conferences

- Advocate for the promotion of EU and national policies for social research and quality of life studies
 - Disseminate the EURORDIS Position Paper on the social challenges faced by people living with rare diseases and their relatives and on the need to integrate rare diseases into existing social policies and services
 - Follow-up of social research projects and dissemination of relevant findings on the socio-economic difficulties faced by people living with rare diseases in Europe
 - Perform preliminary research and action plan for a EURORDISCare 4 Survey on the Social Burden and Financial Burden of Rare Diseases for Patients and Families – concept, research plan, organisation, academic partners, funding – for a possible start in 2015–2016 and implementation & dissemination over three years
- Stimulate the development and the improvement of access to Respite Care Services, Therapeutic Recreation Programmes, Adapted Housing and Resource Centres for Rare Diseases:**
- Maintain and expand EURORDIS website sections for Respite Care Services, Therapeutic Recreation Programmes, Adapted Housing Services, and Resource Centres containing all relevant definitions, information and contacts collected
 - Map existing services and update services' contacts and information (available online)
 - Facilitate the exchange of experiences between services through: the dissemination of a document on consensual guiding principles for Specialised Social Services; the dissemination of case studies on Respite Care Services, Therapeutic Recreation Programmes, Adapted Housing and Resource Centres derived from country visits performed within the EJA; the dissemination of a document on consensual principles for the training of social service providers
 - Advocate for the creation of new Respite Care Services, Therapeutic Recreation Programmes, Adapted Housing Services and Resource Centres through the Policy Fact Sheets, Presentations delivered at the European Conference on Rare Diseases, under Theme 6 – "Beyond Medical Care", and the monitoring of measures adopted in national plans
 - Encourage EURORDIS staff, volunteers and members to volunteer at Summer Camps and members of EURORDIS Round Table of Companies to financially support the Summer Camps or participate as volunteers
 - Organise a workshop on 'Guiding Principles for Social Care in RD (Social Policies and Services)' that will encourage a multi stakeholder group (EUCERD MS representatives, social services, patient groups, national authorities, professional societies, academia, etc.) to brainstorm on Guiding Principles for Social Care in RD and reach consensus on some essential principles

Research, Drugs & Therapies

INVOLVEMENT WITH RESEARCH PUBLICATIONS

- Member of the Editorial Board of OrphaNews Europe**
- Member of the Editorial Board of the Journal Expert Opinion on Orphan Drugs**
- Link with Orphanet Journal of Rare Diseases**

SHAPING AND PROMOTING RESEARCH POLICY SUPPORTING CLINICAL RESEARCH

- Promote Research on Rare Diseases as a Policy and Budget priority at international, EU and national levels with an integrated approach through:**
 - Dissemination of EURORDIS Policy Fact Sheets and Reference Papers on Research
 - Organising a theme in ECRD 2014 Berlin on Research Policy
 - Participation in and support the development of the International Rare Disease Research Consortium (IRDiRC) of which EURORDIS is a member of the Executive Committee, a member and Chair of the

Therapies Scientific Committee and Working Groups on Ethics & Legal Affairs, Registries, Regulatory Affairs, Biomarkers. Take part in all meetings and activities

- Increased participation in the ERA-Net project E-Rare involving Member States
- Support and participation in the 2014 International Congress on Research of Rare and Orphan Diseases "RE(ACT)", organised in Switzerland by the BlackSwan Foundation
- Participation in research policy activities related to national plans for rare diseases (EUROPLAN)

Promote Patient Rare Disease Registries and Data Collection

- Support the development of an EU platform on Rare Disease Registries at the Commission Joint Research Centre
- Disseminate the EPIRARE Book and EURORDIS 10 key principles, EUCERD Recommendation on registries, and Patient's preferred policy scenario on Registries
- Website section on registries
- Workshop at EMM 2014 Berlin

| Participate in the development of new long term Infrastructure projects on Registries, BioBanks and -omics- through:

- Engage EURORDIS in new projects of integration of Rare Diseases European Research infrastructures and study design
- RD-Connect, an integrated platform connecting databases, registries, biobanks and clinical bioinformatics for rare disease research. EURORDIS ensures the involvement of patient organisations in capacity building on registries, biobanking and omics and directly contributes to developing registry, biobanking and omics infrastructures and their integration. EURORDIS ensures a strong interaction and coordination of the RD-CONNECT network with other initiatives within and beyond Europe and the dissemination of the RD-CONNECT project outcomes at the international level (WP1, WP7).
- BBMRI Consortium, ensuring patient representation in its governance
- EuroBioBank, promoting specific agreements between patient organisations and EuroBioBank by providing information, template agreements and advice

SUPPORTING CLINICAL RESEARCH

| Promote good practices for clinical research on rare diseases:

- Promote adoption of the EURORDIS Charter for Collaboration between Patient Organisations and Sponsors of Rare Disease Clinical Trials
- Facilitate the implementation of the Charter with the support of a Mentor
- Collaborate with the ECRIN Project
- Contribute to the revision of the EU Directive on Clinical Trials

| Support specific actions in Rare Cancers:

- Participation in the Rare Cancer Europe network co-founded by EURORDIS
- Collaboration with the European Society of Medical Oncology (ESMO)
- Support coordination between the Commission Expert Group on Rare Diseases and the Commission Expert Group on Cancers to maximise synergies of these two EU policy areas
- Collaborate with the European Cancer Patients Coalition (ECPC) on position paper on Personalised Medicines and Rare Cancer Registries
- Support rare cancer patients community and capacity building through training activities, RareConnect, programmes with European Federations, communication
- Increase relevant visibility of EURORDIS' involvement in rare cancer activities on the EURORDIS website

| Initiate collaboration with the European Network on Clinical Ethics to inform and build capacities of our members. Support their next International conference on clinical ethics in Paris 2014

GAINING KNOWLEDGE ON RARE DISEASE TREATMENTS

| Develop working relations with experts in outcomes research to better integrate patients' views on R&D of medicines, their benefit/risk evaluation, their value for society

Become member of and sign a partnership agreement with the International Society of Pharmacoeconomics and Outcomes research and co-chair its Patient Centred Special Interest Group

The measurement of the impact of medicines in the patient's life is a key component of the reflexion on how to best assess the value of medicines for society.

The objectives of the Patient Centred Special Interest Group would be:

- 1 To determine how to best involve patients and their representatives in all stages of the decision making, on an opinion providing basis, or on a decision making basis, in concordance with the Denver Principles of Patient Advocacy (see reference);
- 2 To analyse expectations and obstacles for a greater involvement of patients in the benefit-risk and the value assessment of medicines;
- 3 To ensure an effective participation of patients' organisations in the creation and development of tools made to measure outcomes patients themselves can measure;
- 4 To identify training needs and opportunities for patients and their representatives in the domains of interest.

| Promote rare disease patient spontaneous reporting on suspected adverse drug reactions of orphan and non-orphan drugs (EURORDIS DIOD project) by:

- Maintaining web pages on EURORDIS website containing explanation why it is important to report adverse drug reactions (ADRs), explanation of the work done by regulatory authorities when receiving reports from patients, explanation of the pharmacovigilance system as a whole, links to reporting sites in Europe, by country/ language
- Providing information to EURORDIS online patient communities and other social media
- Posting announcements in EURORDIS eNews to encourage members to become pharmaco-vigilant and informing them of where to gain knowledge regarding compassionate use
- Presentating the pharmacovigilance system and spontaneous reporting tools to rare disease specific federations
- Organising and participating in sessions on pharmacovigilance in rare disease conferences

- Training of patients advocates through webinars and/or eLearning and/or session at EURORDIS Summer School
- Participating in the Joint Action on Pharmacovigilance Advisory Committee (SCOPE)
- Joining a consortium in response to the Innovative Medicine Initiative call n° 9 "Leveraging Technologies for Pharmacovigilance"

Develop activities within the Drug Information, Transparency and Access Task Force

- Disseminate the results of the off-label use survey 2012 and prepare the extension, publish articles and discuss with regulators
- Prepare possible extension of the survey on off-label use in rare diseases to all other EU Member States and possible creation of a database to document this. Engage dialogue on off-label use with the Patients' and Consumers' Working Party (PCWP), the Committee for Orphan Medicinal Products (COMP), the Pharmacovigilance Risk Assessment Committee (PRAC) and the Committee for Medicinal Products for Human Use (CHMP)
- Continue the activities in parallel to the work plan of the PCWP
- Liaise with EMA and Heads of Medical Agencies for more transparency of compassionate use programmes, support the creation of a compassionate use facilitation group, propose guidelines to companies and Member States for the organisation of compassionate use programmes, publish a Q&A on the subject
- Maintain pages on EURORDIS website containing information on compassionate use programmes with links to the competent department in national agencies,
- Propose a ranking system for the healthcare industry, in order to promote best practices
- Maintain a sub-group of volunteers on specific needs for blind and vision-impaired patients
- Create on-line video tutorials on how to search information on main websites (EudraCT, database on suspected adverse drug reactions, EudraPHARM)

Explore feasibility and resources to conduct research on the use of NATC products (Natural, Alternative, Traditional, Complementary products and also vitamins, food supplements, etc) with rare disease patients in line with our proposal for a research priority in Horizon 2020

PROMOTING DRUG DEVELOPMENT & ACCESS TO TREATMENTS

- Expand activities on medicines development, information and access & prepare a long term representation and leadership of EURORDIS in the EMA Committees:
- Participate in the EMA Committee for Orphan Medicinal Products (COMP) with two representative members and one permanent observer

- Contribute to the EMA Pharmacovigilance and Risk Assessment Committee (PRAC) as external experts for RD Therapies
- Participate in the EMA Paediatric Committee (PDCO) with one representative member and one alternate and one observer
- Participate in the EMA Committee for Advanced Therapies (CAT) with one representative member and one alternate and one observer
- Participate in EMA Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (more commonly known as the Patients' and Consumers' Working Party or PCWP) with one representative member and one alternate and one observer (PCWP)
- Prepare the participation of patient representatives as observers in the Committee for Human Medicinal Products (CHMP) and to contribute to the risk benefit assessment of medicines
- Participate in EMA user testing group of the European Clinical Trials Register (EUCTR), European Database for Suspected Adverse Drug Reactions, European Database of Authorised Medicines
- Identify and support patient experts for Protocol Assistance/Scientific Advice, Scientific Advisory Groups of CHMP
- Support EURORDIS patient representatives in EMA Scientific Committees and Working Parties with the EURORDIS Therapeutic Action Group (TAG) via monthly conference calls and sharing information, agendas, reports, providing mutual support, by discussing main issues
- Review all orphan drug designation applications (ODD), protocol assistance (PA) dossiers, review of designation criteria at the time of marketing authorisation and reports on significant benefit, paediatric investigation plans (PIP) for rare diseases including waivers and deferrals and advanced therapy (gene, cell and tissue engineering) applications
- Review and validate all Public Information on rare disease therapies disseminated by EMA at the time of designation (PSOs) and Marketing Authorisation (EPARs, Package Leaflets, Significant Benefit Public Reports)
- Take action to enhance collaboration between EMA and FDA on rare therapy development beyond orphan drug designation

Investigate and propose solutions to European and national medicines supply shortages:

- Lead the drafting group for a common position of patients' and healthcare professionals' organisations on medicines supply shortages. Create a European/International coalition of interested parties
- Advocate for an improved European regulation of medicines supply shortages, due to both technical and economic factors

| Explore access difficulties patients may have in accessing medicines or other types of medical care:

- Launch the "Access Campaign" to collect feedback from National Alliances, European Federations, DITA task force members, and patients at large on difficulties in accessing treatments in the context of the economic and financial crisis
- Plan and take appropriate action for the future organisation of the 6th EURORDIS Survey on Access to Orphan Drugs in the European Union

| Engage in Health Technology Assessment (HTA) activities & prepare long-term representation and leadership of EURORDIS in HTA:

- Participate in the newly established permanent European HTA Network as member and co-chair of their Stakeholders' Forum (implementation of the EU Directive on Patients' Rights in Cross-Border Care).
- Promote the engagement of patient representatives in its strategic vision, policies and guidelines, methodological approaches and assessment procedures, particularly to address rare diseases challenges
- Collaborate with HTAi on adapted HTA for Rare Diseases. Support and advise the dialogue of National Alliances with HTA Agencies

| Capacity building of our members and volunteers on clinical trials, drug development, EU regulatory processes:

- Organise EURORDIS Summer School 2014 Session in Barcelona in June to train 40 new patients' advocates representing a diversity of diseases and geographical locations
- Collaborate with ECRIN to incorporate 10 non-rare disease patients' representatives from the therapeutic indication areas of nutrition and medical devices in addition to rare diseases
- Continue to improve and contribute to the Training Resources section that currently provides access

to all presentations from the Summer School in downloadable pdf form for presentations, as well as video recordings of the speakers. Online training tools include a preparatory section for the Summer School and the e-learning platform for continued evaluation of learning

- Take part in the DIA EuroMeeting 2014 Vienna in March with speakers, session chairs, contribution to programme committee, a DIA Patient Fellowship Programme for approximately 40 fellows and a Patient Fellows Booth
- Empower our volunteers via regular information and Monthly Therapeutic activity Reports, Call for Volunteers, Support of Drug Information Transparency & Access (DITA) Task Force with more volunteers; Organise one or two workshops of the DITA Task Force annually
- Participate in the "European Patients' Academy on Therapeutic Innovation" (EUPATI or Patients' Academy) from 2012 to 2016, an IMI-funded project gathering a Consortium led by European Patients' Forum (EPF) with other European umbrella patient organisations
- Creation of material for training of audiences. Training material will be freely available on the EUPATI website.
- EURORDIS is also responsible for delivery of the face to face events when required

| Promote dialogue with pharmaceutical & biotech companies involved in rare disease therapy development:

- EURORDIS Round Table of Companies: consolidate membership and organise two workshops in 2014: one in Brussels in February and the second in Barcelona in September
- Expand the direct dialogue with pharmaceutical and biotech companies
- Strengthen the dialogue with EBE-EuropaBio, EFPIA and national pharmaceutical associations

Cross-Cutting Priorities

.....

| Implement EURORDIS Strategy 2010–2015:

- Develop financial orientations 2014–2015–2016
- Improve planning anticipation of major EURORDIS activities such as ECRDs, Membership Meeting, Rare Disease Day, EURORDIS Round Table of Companies Workshops, RareConnect, EURORDISCare, major advocacy campaign, new projects
- Begin Strategic Review of EURORDIS Strategy 2010–2015 for final assessment and preparation of Strategy 2016–2020 in 2015
- Continue collection of EURORDIS Indicators and analysis

| Develop EURORDIS Strategic Partnerships:

- Prepare Strategic Partnership with AFM-Téléthon for 2014–2016 based on Collaborative Agreement 2010–2013 and respective strategies of AFM & EURORDIS
- Assess strategic partnership with NORD (USA) and consolidate common actions based on Strategic Partnership Memorandum signed in 2009, for 2010–2015
- Maintain a Partnership with CORD (Canada), the Japanese Patients Association – JPA (Japan) and establish partnerships with Rare Voices Australia and relevant Russian Organisations in addition to other international patient organisations

- Develop Strategic Partnership with ORPHANET
- Maintain global partnership with DIA
- Develop Strategic Partnership with the European Society for Human Genetics, EuroGentest, International Society of Social Workers, European Institute Women's Health, International Society For Pharmacoeconomics and Outcomes Research ISPOR, Health Technology Assessment International (HTAi), European Society of Medical Oncology (ESMO), European Hospital and Healthcare Federation (HOPE), etc
- Consolidate Partnership with the members of the EURORDIS Round Table of Companies

■ Create the EURORDIS Panel of Experts composed of expert individuals from a range of diverse backgrounds sharing the mission and values of EURORDIS, in order to provide, when needed, a comprehensive approach to complex issues requiring a multidisciplinary approach

■ Increase EC support to EURORDIS activities through:

- Proposal for optimal usage of the Operating Grant as a financial instrument in 3rd Public Health Programme, "Health for Growth"
- Application 2014 for Operating Grant 2015
- Prepare for 2nd Joint Action of the Commission Expert Group on Rare Diseases
- Prepare for the 1st Joint Action of the Commission Expert Group on Cancers
- Application for a Patient Driven Public Health Project in DG Sanco Work Programme 2014–2015 as project leader or partner
- Application for a Patient Driven Health Research Project in DG Research FP 7 Work Programme 2014 or 2015 as project leader
- Study feasibility and apply for grant application to other Programmes in other DGs in support of activities foreseen in EURORDIS Strategy 2015
- Application for patient driven health research project at IMI 2

■ Develop and diversify private funding:

- Maintain overall support level from industry donors spread between ~40 companies and a variety of activities, within EURORDIS Policy of Relationship with Commercial Companies, EMA Policy on Prevention of Conflict of Interest and EAHC rules
- Engage corporate and foundation donors beyond the pharmaceutical industry in supporting EURORDIS' projects & actions. Priority to co-funding of the Joint Actions; RD-Connect; EURORDIS Membership Meeting; the Website & eNews & eLearning & EURORDIS TV & EURORDIS InfoHub in seven languages; the RareConnect project & Social Media;

Support to European Federations & Networks; Patient Surveys

- Organise the EURORDIS Black Pearl Gala Dinner for Rare Disease Day on 25 February 2014 in Brussels and plan Gala Dinner 2015
- Develop a plan for individual donors to be implemented from 2014 & 2015
- Promote in-kind support from private partners (ex: travels, communication tools)

■ Implement the Staff Strategy & Organisation & Evolution 2011–2013

- Complete the new organisation in 3 Units: Governance & Public Affairs, Operations & Knowledge Management, Finance & Support Services
- Develop new team management structure in line with the needs of the organisation and the Strategy 2010–2015
- Create the position of Fundraising Manager
- Create the position of Research & Healthcare Director
- Create position(s) of trainee assistant
- Revise and improve the coordination processes: Operating Grant Steering Committee Meetings (4 per year), All Staff Meetings (3 times a year), Finance Meeting (monthly), Resource Development Meeting (monthly), Monday Meetings, Advocacy & Strategy Meetings (monthly), Editorial Meetings (bimonthly), Management Meeting (monthly)

■ Seek alternative human resources:

- Opportunities to secure seconded staff
- Create EURORDIS Internship Opportunities, unpaid and paid, for up to five interns per year for periods of 1 to 6 months

■ Maintain procedures in the field of Finance, Human Resources and Office Support Services

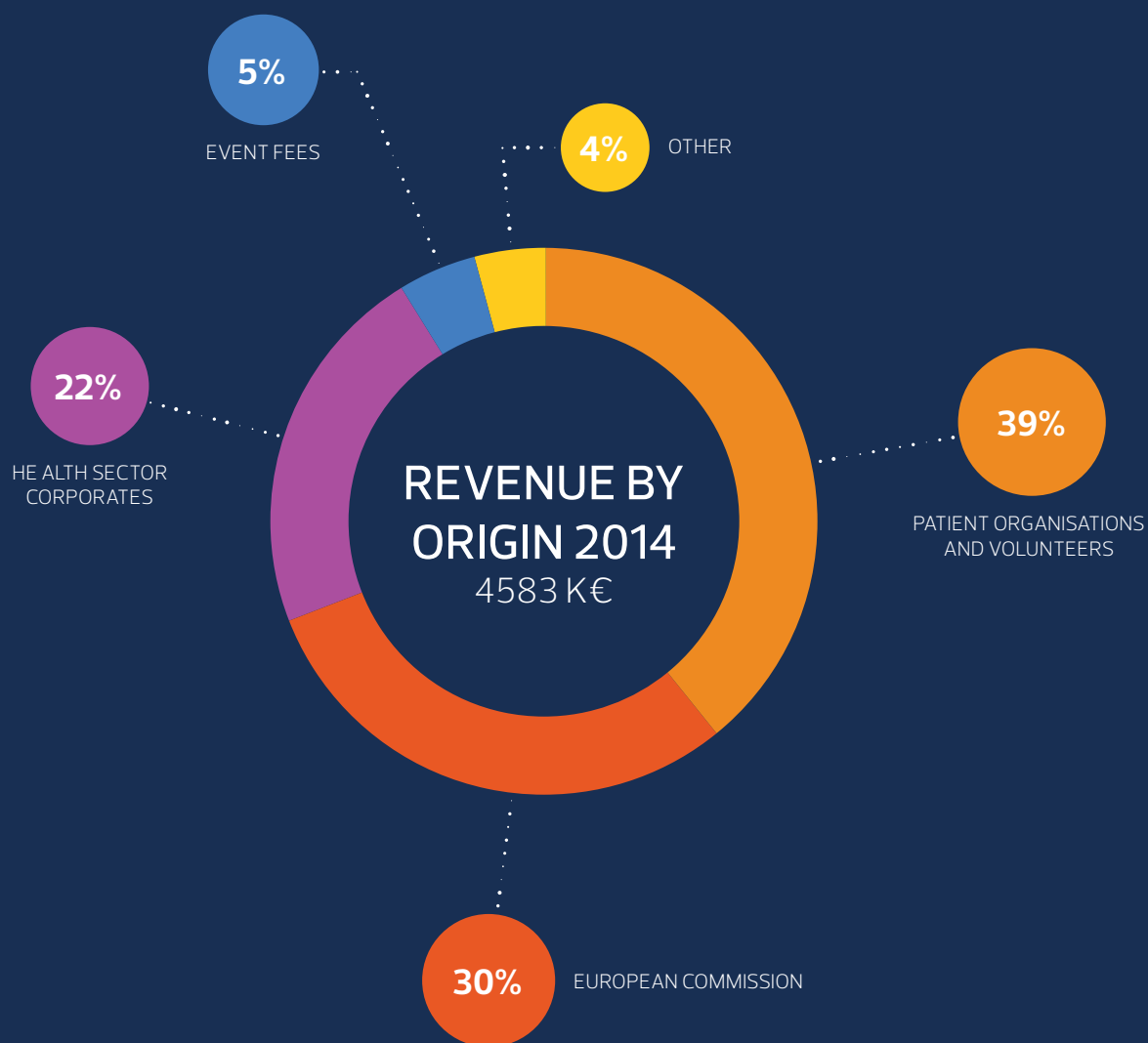
■ Maintain the decentralised structure from Paris (Main Office), Brussels (European Public Affairs), London (EMA), Barcelona (Web Communications & Rare Connect) with integrated operations through work processes, IT standards & intranet, voice & data internet communication

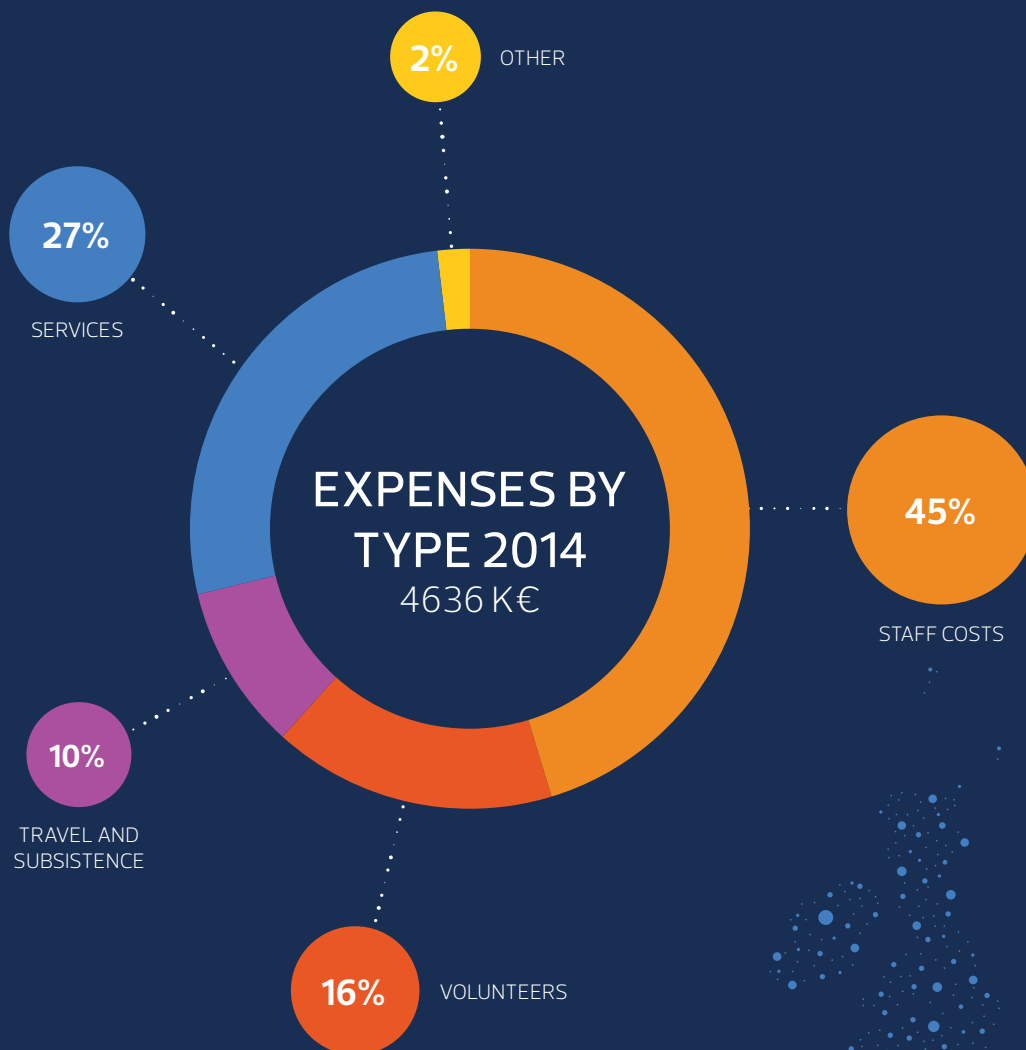
■ Reinforce the Barcelona presence with a contracted office space

■ IT support: equipment, services, virtual office, open to volunteers

■ EURORDIS Contact Database Management fully operational

REVENUE & EXPENSES 2014





GOVERNANCE CHART²⁰¹⁴

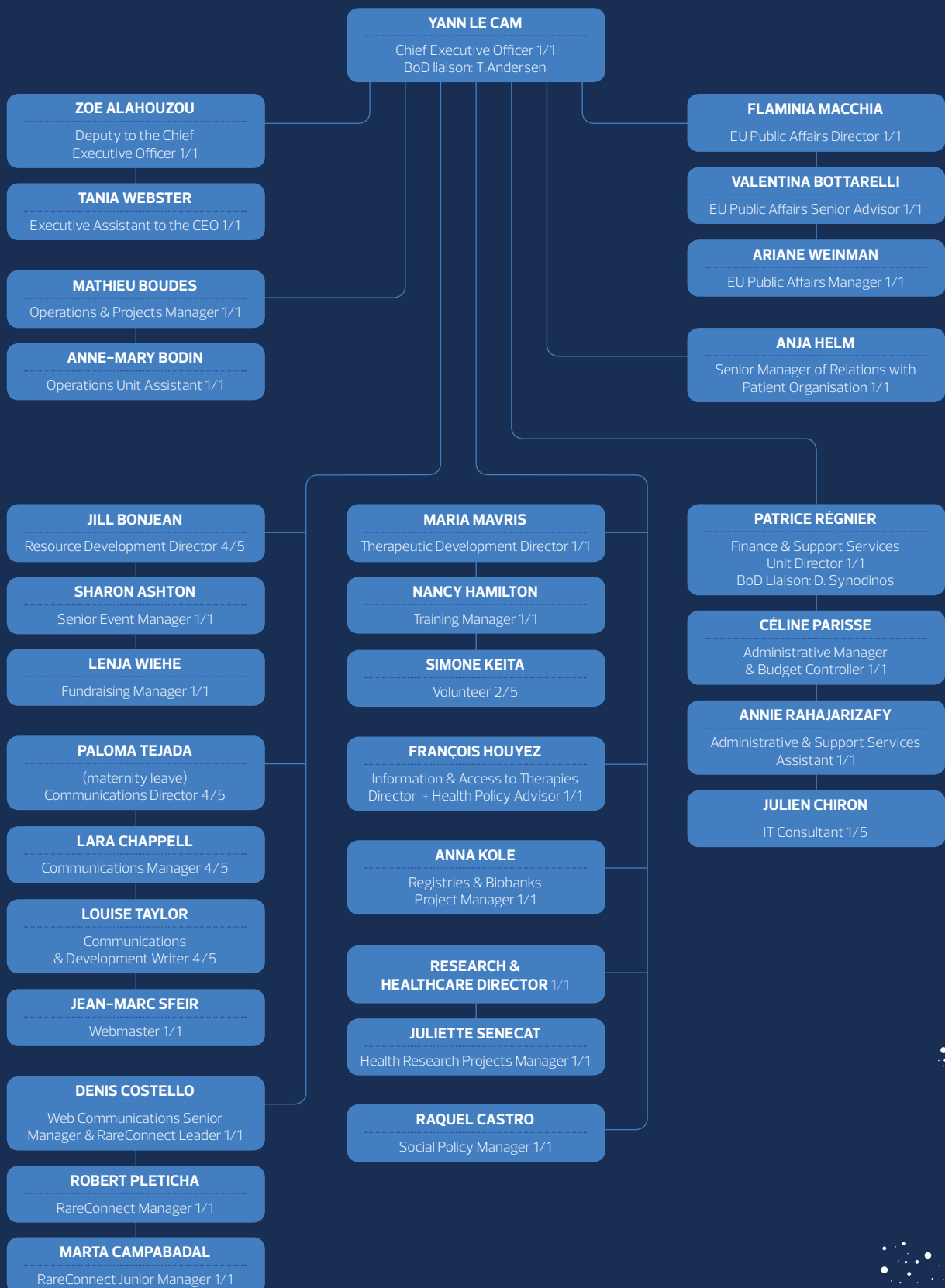


EXTERNAL REPRESENTATION CHART²⁰¹⁴



- GOVERNMENTAL INSTITUTIONS
- NON-GOVERNMENTAL ORGANISATIONS

TEAM CHART 2014





EDITORIAL COMMITTEE
EURORDIS

.....
GRAPHIC DESIGN
www.fabiennail.com
+33(0) 6 6122 26 30
www.emmanuellaunay.com
.....

eurordis.org



EURORDIS
Rare Diseases Europe

European
Organisation
for Rare Diseases

ACTIVITY REPORT ²⁰¹³

& WORKPLAN 2014

PARIS OFFICE

EURORDIS – Plateforme Maladies Rares
96, rue Didot
75014 Paris
France
Tel: +33 (0) 1 56 53 52 10
eurordis@eurordis.org

.....

BRUSSELS OFFICE

EURORDIS
Avenue Louise 149/24
1050 Brussels
Belgium
Tel: + 32 (0) 2 535 75 26

.....

BARCELONA OFFICE

c/o Fundació Dr. Robert
UAB Casa Convalescència
St. Antoni Maria Claret, 171
08041 Barcelona
Spain

eurordis.org

