



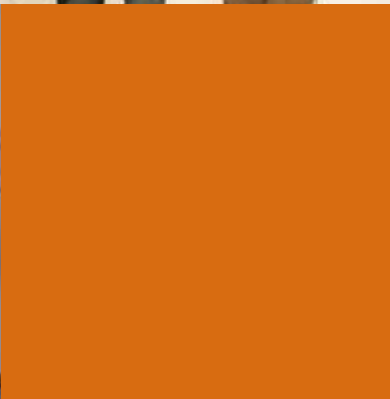
**EURODIS**  
Rare Diseases Europe

EUROPEAN ORGANISATION FOR RARE DISEASES

# Activity Report → 2009



Paris, March 2010



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# Activity Report →2009

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# Foreword

## by the President



*In 2009 EURORDIS focused on promoting an EU policy base which can translate into a better environment for people living with rare diseases in all EU Member states. We can be proud of our achievements.*

*The Council Recommendation on Rare Diseases was adopted in June 2009. This essential policy text adopted unanimously by the Council of Ministers of the 27 Member states, promotes the adoption of national strategies and plans in all EU Member states by December 2013. The Recommendation delineates the main pillars of any national strategy: classification and codification, research, centres of expertise, sharing of expertise, patient empowerment, information and specialised services.*

*This Recommendation follows the adoption, in November 2008, of an EU policy framework: the Commission Communication on Rare Diseases. This “EU policy diptych” was initiated under the EU Presidencies of Luxembourg in 2005 and Portugal in 2007, and supported by the EU Presidencies of France in 2008 and the Czech Republic in 2009. During the last five years, EURORDIS has relentlessly fed the political momentum, voiced the patient needs and expectations and contributed with concrete proposals. The key success factors have been the high mobilisation of the rare disease community, as well as the high quality dialogue and intense partnering between all stakeholders which has enabled to call for the same policies and actions.*

*With this framework in place, we are where we wanted to be in order to take our cause through the next steps and in particular towards the implementation of national strategies and plans in all EU Member states by 2013. For this next phase, shaping a better policy environment at national and local levels, we will act from three platforms: the EURORDIS Council of National Alliances, the EuroPlan project involving 24 Member states together with EURORDIS, and the new EU Committee of Experts on Rare Diseases. We will also rely on our committed staff and volunteers to further integrate our activities in public affairs, communications, outreach, capacity building and networking, with the ones in each area of action, such as research policy and networking, drug development and access, centres of expertise and standard of care, as well as information to patients.*

*Our endeavours at raising political awareness are now very much linked to our efforts at raising public awareness. The Rare Disease Day 2009 and 2010 were even more successful than the first one in 2008. Sharing common tools and a common message “Patient Care: a Public Affair!” and “Patients & Researchers: Partners for Life!”.*

**Terkel Andersen**  
President



# Foreword

## by the Chief Executive Officer

*EURORDIS is recognised for its vision, its good governance and management, its independence and wise financial structures as much as for its achievements. It is also renowned for its solid community base and for its well established and listened to European voice. This is the ground from which we can address the challenges ahead for the next five years.*

*EURORDIS legitimacy is rooted in a robust community base. At the end of 2009, EURORDIS had 406 members in 42 countries, amongst which 25 EU Member States. This is 50 more members in three more countries than last year. The EURORDIS Council of National Alliances is now composed of 20 alliances and the Council of European Federations brings together 30 European Rare Disease Federations and Networks.*

*EURORDIS credibility is the result of 10 years of efforts. EURORDIS' empowered voice is based on expertise, professionalism, determination, independence and transparency.*

*In 2009, over 100 volunteers from all over Europe and 19 full time equivalent staff operating from the Paris and Brussels' offices have contributed in all areas of EURORDIS activities. The shared values, vision, objectives, work processes of this group of highly motivated and committed individuals (the vast majority of which are living with a rare disease) are what makes our organisation so special and good at delivering.*

*In 2009, EURORDIS has reached a sound financial situation in terms of structure of revenues, reserves and cash flow. EURORDIS revenues are not sufficient to carry on recurrent activities and take on new challenges, over the next five years, but they provide a base from which to grow and diversify our sources of revenue to better serve our mission.*

*EURORDIS has conducted an in-depth strategic and organisational review to define its new Strategy 2010-2015. We know where we are, where we want to be five years from now, and how to get there.*

*In 2009, EURORDIS and NORD have signed a Strategic Partnership in order to support our actions on both sides of the Atlantic and to leverage our efforts on an increasingly global scale. We will develop partnerships with key players in Europe so as to join forces and enhance our synergies while optimising our limited resources in order to address patients' needs.*

*Yann Le Cam*  
Chief Executive Officer

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.

It 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), the European Commission, corporate foundations and the health industry.

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



# Eurordis in Brief



## → Key figures 2009 :

- 406 member patient organisations
- 42 countries (25 EU countries)
- 20 national alliances
- More than 700 patient groups represented
- More than 1000 rare diseases represented
- 22 staff members
- 104 volunteers
- Revenue : 2,862,000 €
- EURORDIS participated in 43 conferences, congresses and symposia

## Our mission →

### Eurordis' mission is:

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.

### To this end, EURORDIS undertakes activities on behalf of its members, notably in favour of:

- **Empowering** rare disease patient groups;
- **Advocating** rare diseases as a public health issue;
- **Raising public rare disease awareness**, and also that of national and international institutions;
- Improving **access to information, treatment, care, and support** for people living with rare diseases;
- Encouraging **good practices** in relation to these;
- Promoting scientific and clinical rare disease **research**;
- Developing rare disease **treatments** and **orphan drugs**;
- Improving **quality of life** through patient support, social, welfare, and educational services.

# Our Strategic Approach 2007-2010

## Rare Diseases: a priority visible on the European agenda

- Promote rare diseases as a public health priority at EU and national levels
- Organise the European Rare Disease Conference 2010 Krakow
- Promote an enhanced EU policy framework with a Commission Communication and a Council Recommendation on Rare Diseases
- Promote national plans on rare diseases in EU Member states
- Promote rare diseases as a global public health priority
- Raise rare disease awareness through Rare Disease Day

## Consolidate and empower the rare disease patient community

- Broaden the patient group membership base
- Focus on new and future Member States
- Bring up the network of National Rare Disease Alliances to a more active level
- Create the network of European Rare Disease specific Federations
- Create a new version of EURORDIS Website and a social media platform
- Identify, involve and support volunteers in more activities
- Organise capacity building and training sessions for patient representatives

## Shape the European environment for better Information, Care and Drug development for Rare Disease patients and families

- Promote access to quality care
- Promote access to quality information and support services
- Promote development of and access to orphan drugs, paediatric drugs and advanced therapies
- Address bottlenecks in therapy development through patient-driven European research infrastructures
- Bridge the gap between patient needs and research

## Achieve cross-cutting priorities

- Perform EURORDIS Strategic Review 2010-2015 involving all members and relevant stakeholders
- Include web communications in all EURORDIS activities
- Develop office support services for better quality work and increased efficiency
- Increase EU support to EURORDIS projects and other rare disease activities
- Reinforce the current funding base from the French Muscular Dystrophy Association (AFM), members and the health industry
- Develop private funding and partnerships with corporations and foundations outside the health sector, from the EU and the US



# → Highlights 2009

## Advocacy

- Adoption of the Council Recommendation on a European action in the field of rare diseases (CRRD)
- Implementation of the Commission Communication "Rare Diseases: Europe's Challenges" (CCRD)
- Response to the EC consultation on the creation of the Committee of Experts on Rare Diseases (EU CERD)
- Contribution to the development of Indicators on the assessment of National Plans and Recommendations on how to develop a National Plan as part of the EuroPlan project
- Promoting the proposal for the establishment of a Working Party for European collaboration towards a common scientific assessment of the clinical added value of orphan drugs (CAVOD)

## Health Policy & Health Care Services

- Registration launched for the European Conference on Rare Diseases, Krakow 2010
- 6 new Play Decide Games within the POLKA Project
- EURORDIS Declaration on Centres of Expertise and European Reference Networks, launched for Rare Disease Day 2009; together with the publication of the book "The Voice of 12.000 Patients"
- Best practice guidelines for Respite Care Services and Therapeutic Recreation Programmes

## Research, Drugs & Therapies

- Creation of a Therapeutic Action Group to coordinate the activities of 8 patient representatives at EMA Committees and Working Groups (COMP, PDCO, PCWP) as well as EURORDIS Task Forces
- Second "EURORDIS Summer School" for capacity building of patient representatives in aspects of clinical trials and drug development - 39 fellows attended
- 2 successful workshops of the EURORDIS Round Table of Companies
- 2 new members in the EURORDIS Round Table of Companies (total 33 members)

- Survey on role and priorities of rare disease patient organisations in research
- 420,000 rare disease samples (DNA, Cell and Tissue) were available across the EuroBioBank Network in 2009 - 8513 samples collected and 6849 samples distributed

## Networking & Information

- Second Rare Disease Day, coordinated by EURORDIS and involving 30 countries
- Launching of EURORDIS website with new design and new functions
- New on-line patient community portal
- New Rare Diseases Blog
- More members (+50) in more countries (+3)
- Council of National Alliances, 2 workshops, 20 members
- European Network of Rare Disease European Federations, 2 workshops, 30 members
- Nearly 200 participants attended the Membership Meeting 2009 Athens
- More newsletter subscribers (+20%) 7171 total subscribers
- More visits to website (+20%) 19 800 visits/month
- 5 new members applied to the European Network of Help Lines in 2009, bringing total number to 10 Help Lines

## Governance, Organisation & Funding

- Memorandum of Understanding with NORD for a 5-year strategic partnership
- 4 meetings of the Board of Directors and 4 meetings of the Board of Officers
- New grant from the European Commission DG Health and Consumers for recurrent activities (OPERA 2009)
- 4 renewed project applications





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## ADVOCATING FOR PATIENTS

EURORDIS represents patients within European government institutions and advocates for policies which address the needs of patients and their families. We consult our membership and other stakeholders extensively when developing each advocacy action.

# Advocacy



### Sustaining Rare Diseases as an EU Public Health Priority

#### Council Recommendation on a European action in the field of rare diseases - adopted in June 2009

Work with all relevant stakeholders towards the adoption of the Council Recommendation on a European Action in the field of Rare Diseases (CRRD), with special emphasis on the adoption of a national plan or strategy on rare diseases in each Member State of the European Union.

#### Commission Communication "Rare Diseases: Europe's challenges"

Contribution to the creation of the road map for the implementation of the Commission Communication "Rare Diseases: Europe's challenges" (CCRD), working very closely with the European Commission. Identification of main priorities from the rare disease patients' perspective.

Drafting of an article on the Commission Communication, published by the European Patients' Forum (EPF), to increase awareness on recent major steps forward for the EU rare disease community at European level.

#### EU Public Health Programme

Advocacy activities towards officials of the European Commission's Directorate General for Health and Consumers, in order to keep an ▶

- ▶ acceptable level of funding for rare disease-related activities and projects, including operating grants for patient groups, both for new grants and for the renewal of previous ones. Special mention on the budget dedicated to the renewal of Operating Grants in the Work Programme for 2010.

#### EU Committee of Experts on Rare Diseases

Response to the European Commission's consultation on the creation of an EU Committee of Experts on Rare Diseases (EU CERD), as well as advocacy activities in favour of its creation and advice for the appropriate functioning of the Committee. Specific advocacy work concerning the composition of the EU CERD and EURORDIS' proposal of members and substitutes from the patients' side.

### Making Rare Diseases a Public Health Priority in all Member States

#### European Project for Rare Diseases National Plans Development (EuroPlan)

Partner in the EuroPlan Project (2008-2011) to work with National Rare Disease Alliances to promote best practices and measures through national conferences on rare diseases, and develop common minimum recommendations for national strategies and action plans. ▶

- ▶ Contribution to the development of two of the main project deliverables: Indicators on the assessment and the implementation of National Plans, and Recommendations on how to develop a National Plan.

Support to the organisation of National Conferences in 16 countries, through meetings with Advisors in each participating country.

Exchange of information and networking about National Plans for Rare Diseases and EuroPlan in particular, at the Council of National Alliances (May and November 2009).

### Advocacy support to National Plans for Rare Diseases

Direct participation to the evaluation of the first French National Plan on Rare Diseases and to the drafting of the second French Plan.

Particular indirect support to National Plans on Rare Diseases in UK, Ireland, Denmark, as well as Romania, Greece, Germany, Spain and Italy.

EURORDIS has started a series of Fact Sheets to empower patient advocates at EU, National and local levels. The first four are on: Centres of Expertise, European Reference Networks, National Rare Disease Help lines and European Network of Rare Disease Help lines.

## Rare Diseases: An International Public Health Priority

As part of their Strategic Partnership EURORDIS and the National Organization for Rare Disorders (NORD) have started to collaborate to promote rare diseases as an international public health priority by developing a Joint Paper on this topic and expanding Rare Disease Day to a global scale.

EURORDIS also participated in the International Conference for Rare Diseases and Orphan Drugs (ICORD) in Rome.

## Improving Access to Orphan Drugs

### EU Orphan Drug Policy

Promotion of EURORDIS Statement: "Orphan drugs: rising to the challenge to ensure a better future for 30 million patients in Europe" (September 2009) in different forums, when possible and relevant. The paper uses ▶

- ▶ mathematical models to predict the number of orphan drugs that will enter the market in the next ten years and their economic impact.

### Clinical Added Value of Orphan Drugs (CAVOD)

Promotion of EURORDIS' proposal for the establishment of a Working Party for European collaboration toward common scientific assessment of the clinical added value of orphan drugs (CAVOD), with advocacy activities directed towards the pharmaceutical companies, the European Commission and the European Medicines Agency.

### Charter for Clinical Trials in Rare Diseases

Promotion amongst patient groups, of the Charter for Clinical Trials in Rare Diseases to regulate the relationship between a clinical trial promoter and the patient organisations representing the disease concerned by the study.

Launched reflection process on the revision of the Clinical Charter Directives within EURORDIS membership and participation to meetings with the European Commission as expert group.

### Drugs marketed for common indication and not for rare indications

Work done on the issue of access to drugs marketed for common indication and not for rare indications. Liaised with relevant patient groups and patient representatives, as well as with companies for concerned products.

## Improving Access to Quality Care

### Centres of Expertise and European Reference Networks

Contribution to the adoption of the policy on European Reference Networks (ERN) of Centres of Expertise within the Directive on Patients' Rights to Cross-border Healthcare.

Launching of the EURORDIS Declaration on Centres of Expertise & European Reference Networks, for Rare Disease Day 2009.

Presentation and hand over to the ERNs, of the EURORDIS Charter for Good Practice Collaboration between Patient Groups and Centres of Expertise & European Reference Networks.

Encourage patient involvement in ERNs by starting discussions with the Council of European Federations (CEF Workshops May and December 2009).

Partner in the European Network on Rare Paediatric Neurological Diseases (nEUroped) 2008-2011.

Participation in the Committee of designation of Centres of Expertise in France.

### Promoting Cross-Border Healthcare and Patient Mobility

Specific advocacy work towards Members of the European Parliament to support amendments in favour of the "exception" for rare disease patients who should not be obliged to seek prior authorisation before being referred to a specialist outside their country of affiliation.

Advocacy actions to remove the obligation for patients to have to pay in advance healthcare purchased abroad and then be reimbursed at the level of national reimbursement.

EURORDIS has advocated this position at crucial times towards the UK Permanent Representation in Brussels on the John Bowis Report on Cross-border Healthcare and has done a great deal of internal explanatory work, liaising with MEP John Bowis and MEP Françoise Grossetête's office at the European Parliament.

- ▶ to collect relevant arguments in favour of targeted and increased investment in rare disease research, at national and EU level.

### Work to promote rare disease registries and databases

Organisation of a Workshop on registries and databases at the Annual Membership Meeting, Athens in May 2009.

Participation to the organisation of the European Platform for Patients' Organisations, Science and Industry (EPPOSI) Workshop on Registries for Rare Disorders in March 2009.

Participation to the drafting of the Rare Disease Task Force Report on patient registries and databases in the field of rare diseases: technical, legal and ethical issues.

## Genetic testing and Newborn Screening

As part of the Patients' Consensus Preferred Policy Scenarii for Rare Diseases project (POLKA), promotion of deliberative patients' debates - *Play Decide* games on:

- Neonatal screening
- Diagnosis, Information to the Patient and Genetic Counselling

Participation in the future European Commission's Directorate General for Health and Consumers project on newborn screening led by the Italian Health Ministry, by bringing the rare disease patient experience and needs.

## Bridging Patients and Research

### European Workshop on Rare Disease Research

Work leading up to European Workshop on Rare Disease Research for Rare Disease Day 2010. The event will be co-organised by EURORDIS and E-RARE in partnership with the European Commission, Orphanet and EuroPlan. The Workshop will aim at identifying the future priorities in rare disease research, both from the researcher and patient perspective, as well as ensuring a better collaboration of all interested parties.

### Survey on patients' role and priorities in rare disease research

EURORDIS conducted a survey on the role and priorities of patients in rare disease research. The results of this survey will help to acknowledge the role of patient organisations in research, and will contribute to shaping the future research agenda of the European Union and Member States.

### Discussion Paper on "Why Invest in Rare Disease Research"

Drafting of a Discussion Paper arguing in favour of investing in rare disease research. Consultation ▶

## Other issues

### Information to Patients

Follow-up of EU regulation regarding the provision of information to patients on medicinal products. Participation in various meetings and discussions to present the specific view of rare disease patients, including at the European Federation of Pharmaceutical Industries and Associations (EFPIA) Think Tank meetings.

Meeting at the European Parliament with the Environment, Public Health and Food Safety Committee (ENVI) (Rapporteur MEP Christofer Fjellner).

Exchange of information and views on this issue with the European Cancer Patient Coalition (ECPC), ▶

- ▶ Alzheimer Europe, European Patients' Forum (EPF) and other patient representatives.

### ■ Animal Experimentation

Participation to discussions on this issue from the patient's perspective in several occasions (various meetings at the EFPIA Think Tank).

Co-signature, together with other patients groups, of a letter on Animal Welfare sent to the European Parliament, especially to the members of the Committee on Agriculture and Rural Development (AGRI).

### ■ Rare Cancers

Advocacy activity on rare cancers, mainly towards industry and patient groups. Specific support and advice to rare cancer patient groups to gain access to cancer orphan drugs.

Promotion of rare cancers as a major topic at the European Platform for Patients' Organisations, Science and Industry (EPPOSI) 10th Workshop on Partnering for Rare Diseases Therapy Development in Brussels in October 2009.

Dialogue with the European Cancer Patient Coalition.

### ■ Organ Donation and Transplantation

Follow-up on the legislative progress of the proposal for an EU Directive on Organ Donation and Transplantation (ODT). Regular updates to the EURORDIS Expert Group on ODT.

### ■ Blood and Plasma Derived Products

Letter sent to the European Commission with questions about the future availability of plasma-derived medicinal products in the European Union.

## → Council Recommendation on a European action in the field of rare diseases

A European strategy that calls upon Member States to implement national plans for rare diseases, before the end of 2013, was adopted by the Council of Health Ministers of the EU in June 2009.

The Council Recommendation is important because it calls for concerted action at EU and national level in order to:

- Ensure that rare diseases are adequately coded and classified
- Enhance research in the field of rare diseases
- Identify Centres of Expertise by the end of 2013 and foster their participation into European Reference Networks
- Support the pooling of expertise at European level
- Share assessments on the clinical added value of orphan drugs
- Foster patient empowerment by involving patients and their representatives at all stages of the decision-making process
- Ensure the sustainability of infrastructures developed for rare diseases

This adoption marks the culmination of a series of legislative declaratory acts which have paved the way towards the recognition of rare diseases as a public health priority and as an area of unique European added-value for Community action.

EURORDIS has been at the centre of this process, voicing the patients' demands for a European policy framework for rare diseases, every step of the way. Starting with the successful *Public Consultation on Rare Diseases* in November 2007, followed by the adoption of the *Commission Communication on Rare Diseases*, in November 2008 and the adoption of a *Council Recommendation on a European Action in the field of Rare Diseases* in 2009, each step has demonstrated the vital importance of EU action, as well as cooperation between Member States.



## PROMOTING RARE DISEASE HEALTH POLICY DEVELOPMENT

EURORDIS pushes for the adoption and actively supports the implementation of coherent and coordinated rare disease strategies at the European and national level. We organise the largest and most influential rare disease policy conference in Europe.

# Health Policy & Health Care Services



### Patients' Consensus on Preferred Policy Scenarios for Rare Diseases project – POLKA

Implementation of the POLKA project, which started in 2008. The central idea of this project is to foster the opinion of patient representatives on future European policies for rare diseases, or to collect their views on existing ones. In short: "Shaping the future by empowering patients with adequate advocacy tools". The project rests on 3 pillars: Deliberative patients' debates; European Reference Networks and Centres of Expertise for rare diseases; and 5th European Conference on Rare Diseases.

#### Deliberative patients' debates – *Play Decide*

Creation of six new *Play Decide* games on the following topics of particular importance to rare diseases: neonatal screening, cross border health care, the cost of care (the case of orphan drugs), stem cell research, diagnosis information and counselling, pre-implantation genetic diagnosis. The games have been translated into 21 languages (this is the first time EURORDIS has decided to develop materials in most European languages). We also contributed to the overhaul of the dedicated website: [www.playdecide.eu/getinvolved/projects/](http://www.playdecide.eu/getinvolved/projects/)

#### European Reference Networks and Centres of Expertise for rare diseases

Foster the involvement of patient representatives in shaping the policy of European Reference

Networks of Centres of Expertise, and offer continuous advice to the European Commission on its policy regarding these networks.

Partner in the European Network on Rare Paediatric Neurological Diseases (nEUroped) from 2008-2011, in order to involve patients in the project and support them in establishing a European network of patients' organisation and families of children affected by rare neurological diseases of the child. A Patient Network Meeting was held on 21 March, 2009 to continue the empowerment of patients affected by rare paediatric neurological diseases by integrating their input in this European Reference Network.

#### 5th European Conference on Rare Diseases, ECRD Krakow 2010: "From Policies to Effective Services for Patients"

Set up of the Programme Committee and identification of the eight themes, which will be debated at the European Conference on Rare Diseases (ECRD).

Set up of the Local Organising Committee and hiring of local events management company. Creation of the official ECRD website ([www.rare-diseases.eu](http://www.rare-diseases.eu))

Launching of registration in March 2009, as well as a Fellowship Programme to support the participation of patient representatives from Central and Eastern Europe. >



## Placing rare disease patients at the heart of the healthcare system

EURORDIS conducts surveys and manages projects that aim at giving patients a voice in the health care policy that affects them. Based on these, we propose adapted organisational models of healthcare and social services, namely Centres of Expertise and European Reference Networks, genetic testing and neonatal screening.

### The Book "The Voice of 12,000 Patients" (EurordisCare 2&3)

Publication of a book which presents the conclusions of the EurordisCare 2 and EurordisCare 3 surveys on the experience and expectations on diagnoses and access to care of 12,000 patients representing 18 rare diseases and 24 European countries.

Over 4 400 hard copies were distributed during 2009 and electronic copies are available on the [EURORDIS](#) website. The Executive Summary of the book, also available for download on the website, has been translated into 6 languages.

### Training of health care professionals

Six-hour training session about the role of patient organisations in health care policy to Masters students of the University of Paris 13 (Public Health, topic Health and Education).

Two-hour training session every year on "Rare Diseases, definition and specificity from the patient's point of view" to third year medical students of the University of Necker-Cochin (Paris Descartes).

## Support services to patients

EURORDIS promotes the implementation of services adapted to the situation and special needs of people living with rare diseases. We facilitate the networking of Respite Care Services and Therapeutic Recreation Programmes in Europe, promote the sharing of good practices, and provide information on these services.

### European network of respite care services

This activity is a continuation of the work that was done during the Rare Disease Patient Solidarity Project (RAPSODY) on increasing the quantity and quality of Respite Care Services (RCS) that exist around Europe.

More services have been added to the Rapsodyonline ([www.rapsodyonline.eu](http://www.rapsodyonline.eu)) tool. There are now over 15 RCS featured on the site which are searchable by patients looking for services in their area.

The Best Practice Guidelines have been updated and modified to reflect the range of different types of respite services (residential, domiciliary and emergency) that exist today. All listed Services are being invited to comment or modify the text by means of a wiki that has been set up on the Rapsodyonline website.

## Therapeutic recreation programmes

This activity is a continuation of the work that was done during the Rare Disease Patient Solidarity Project (RAPSODY) to increase the quantity and quality of Therapeutic Recreation Programmes (TRP) that exist around Europe today. EURORDIS continues to identify TRPs around Europe which it then adds to the Rapsodyonline cartography listing. There are now over 40 TRP services listed on the site. Best Practice Recommendations have been consolidated into a working paper that will be open for comments.

## The Voice of 12,000 Patients

This EURORDIS publication was officially launched on Rare Disease Day 2009 by EU Commissioner for Health Androula Vassilou. The book presents the conclusions of the EurordisCare 2 and EurordisCare 3 surveys on the experience and expectations on diagnoses and access to care of 12,000 patients representing 18 rare diseases and 24 European countries. It details the methodology, the overall results, results by country and by disease, and how the surveys can contribute to the development of policies and actions in favour of better diagnosis and care for rare disease patients.

The main conclusions of the book are intended to serve help the future of EU policy regarding European Reference Networks and Centres of Expertise for rare diseases. The book is an important element of the work to promote the EURORDIS Declaration for Centres of Expertise & European Reference Networks and the Eurordis Charter for Good Practice Collaboration between Patient Groups and Centres of Expertise & European Reference Networks.

An electronic version is available on the website [www.eurordis.org](http://www.eurordis.org) and on the EU Public Health portal [http://ec.europa.eu/health/rare\\_diseases/publications/index\\_en.htm](http://ec.europa.eu/health/rare_diseases/publications/index_en.htm) and on Google books <http://books.google.com/books?id=13g4hrVLBnoC>





## SHAPING RESEARCH POLICY

EURORDIS contributes to the promotion and maintenance of rare diseases as a priority in EU research policy and funding schemes.

# → Research, Drugs & Therapies

## Survey on role and priorities of rare disease patient organisations in rare disease research

Survey on patients' role and expectations in rare disease research, conducted amongst 300 patient organisations, representing 110 rare diseases in 29 European countries. The results of this survey will help to acknowledge the role of patient organisations in research, and will contribute to shaping the future research agenda of the European Union. The results will also help national patient alliances advocate for more and better research for rare diseases at the national level.

## → Supporting clinical research

Research advances in the field of rare diseases could not be possible without patient participation in clinical trials, registries and bio-banks. EURORDIS has created and maintains the European network of DNA, Cell and Tissue Banks for Rare Diseases (EuroBioBank). We represent the needs of patients in European research networks and empower patients in clinical research activities.

## The EuroBioBank Network ([www.eurobiobank.org](http://www.eurobiobank.org))

A total of over 420,000 rare disease samples (DNA, Cell and Tissue) were available across the EuroBioBank Network in 2009. An indicator of the network's dynamism is the number of samples exchanged: 8513 samples collected and 6849 samples distributed. 27 publications acknowledging EuroBioBank have resulted from scientific work performed with samples from the network in 2009.

In 2009, the EuroBioBank network, as partner of the TREAT-NMD Network of Excellence (Translational Research in Europe – Assessment and Treatment of Neuromuscular diseases [www.treat-nmd.eu](http://www.treat-nmd.eu)), organised three specialised training sessions in muscle cell cultures at the Friedrich-Baur Institut/MTCC in Munich, a member of EuroBioBank.

In addition, as coordinator of the EuroBioBank network, EURORDIS pursues its collaboration with the Biobanking and Biomolecular Resources Research Infrastructure (BBMRI) and in particular proposed EuroBioBank to be part of the future BBMRI prototype. Furthermore, EURORDIS has brought the voice of rare disease patients to the BBMRI network by joining the BBMRI Stakeholders' Forum.



## Collaboration with other rare disease research networks

Patient representation and collaboration in the following rare disease research networks supported by the European Commission:

- **Clinigene**

The European network for the Advancement of Clinical Gene Transfer and Therapy is a Network of Excellence funded by the EC-6th Framework Programme. The overall objective of the network is to integrate multidisciplinary research and development in gene therapy. EURORDIS is a member of the Ethical Review Board.

- **ECRIN**

The European Clinical Research Infrastructures Network is a network of excellence gathering the main public clinical research centres in order to share good practices and methodologies to facilitate international clinical trials in all fields. EURORDIS has been invited to join as patient representative.

- **E-RARE**

E-Rare is the Era-Net project that has been established to develop synergies between 8 national research programmes on rare diseases in the European Union, and to set up a common research policy on rare diseases. EURORDIS is a member of the External Advisory Board.

- **nEUroped**

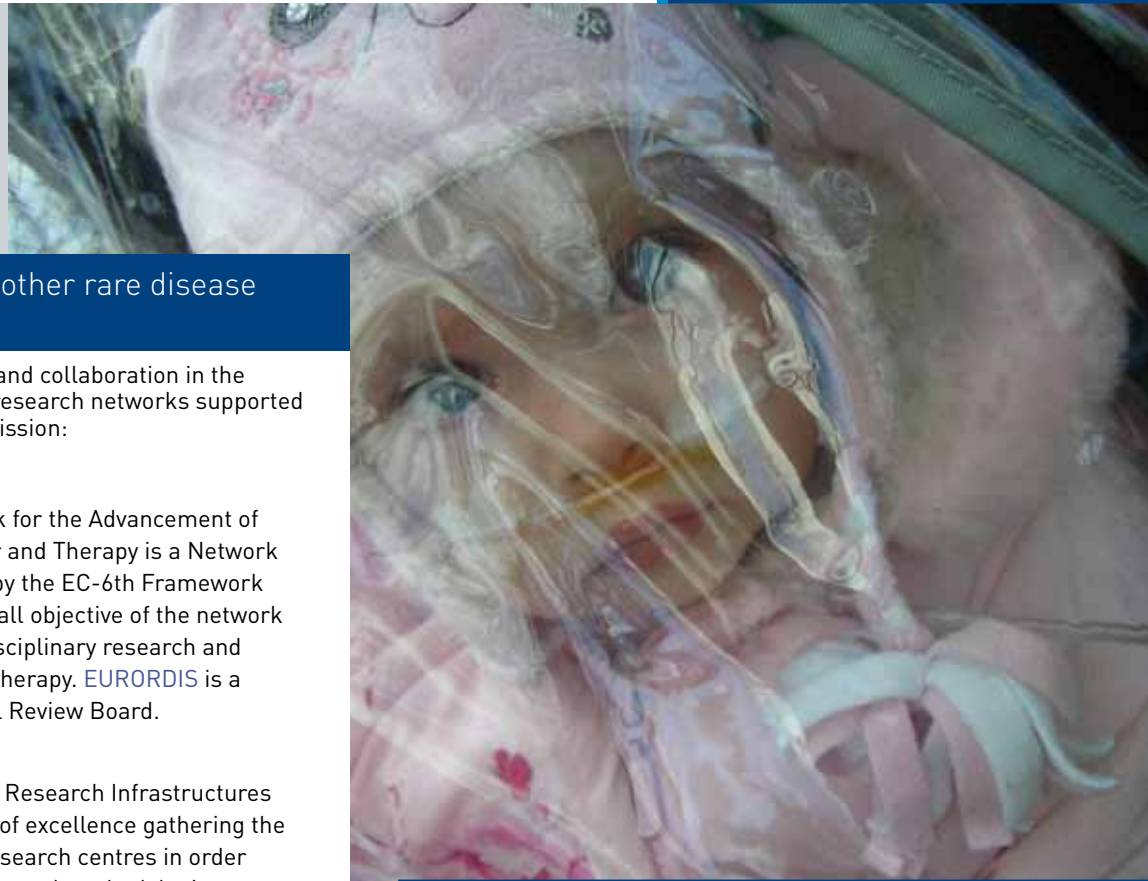
The European Network on Rare Paediatric Neurological Diseases (nEUroped) project aims to develop a network of communication and information sharing across the field of rare nervous system disorders in children characterised by paroxysmal attacks. EURORDIS participates as a full network partner.

- **STEM-HD**

EURORDIS is a member of the Advisory Board of the Stem cells for therapeutics and exploration of mechanisms in Huntington's disease consortium.

- **TREAT-NMD**

TREAT-NMD is a major initiative in the neuromuscular field that is creating the infrastructure and the tools to ensure that the most promising new therapies reach patients as quickly as possible. As a partner of the TREAT-NMD Network of Excellence, EURORDIS represents the EuroBioBank network and the biobanking activities for the project.



## EURORDIS Survey "European Rare Disease Patient Groups in Research: current role and priorities for the future"

Conducted by EURORDIS between October and November 2009 this survey aimed to find out, in what ways and to what extent, patient organisations support research. The survey was designed to find out which are the research areas that should be given priority to, from the patient's perspective.

772 rare disease patient organisations received an invitation to fill out the on-line questionnaire, which was available in 6 languages (English, French, German, Italian, Spanish and Hungarian). In total, EURORDIS received 309 valid responses from members and non-members (40% response rate); representing 110 rare diseases and 1.3 million patients in 29 European countries.

### Key findings:

- 37% of POs funded research in the last 5 years
- Amongst them, half spend more than 30 000 euros yearly and one quarter spends more than 112 000 euros a year.
- Some devote more than 100% of their budget, which means they organise fundraisers specifically for research.
- POs who are 10 years or older spent about half of their budget on research.
- Patient organisations support research mainly through actions aiming at creating links between patients, researchers and physicians.
- 1 in 2 POs support research by helping to identify patients to participate in clinical trials or by providing information and counselling for potential participants in clinical trials.
- Almost 1 in 2 POs collaborated in clinical trial design and helped define research projects by highlighting patients' needs and expectations.
- 1 in 3 POs participate in scientific committees within institutions, which reveals that their legitimacy is increasingly acknowledged and that their desire to support research is also political.
- Patients say the highest priority for the allocation of public funds should be clinical research (therapeutic and diagnosis). Nevertheless, patient groups fund mostly basic research and epidemiological studies.

This work was undertaken in collaboration with the Centre de Sociologie de l'Innovation (Ecole des Mines, Paris, France)

## Promoting drug development & access to treatments

EURORDIS intervenes in the orphan drug, advanced therapies and paediatric-use regulatory process and works with industry to speed up the development and availability of treatments.

We promote transparent and quality information on clinical trials and medicines for patients.

## Patient representatives at the European Medicines Agency

Patient representation in the following European Medicines Agency (EMA) Committees:

- Committee for Orphan Medicinal Products (COMP): 114 orphan drugs designated, 12 marketed in 2009
- Paediatric Committee (PDCO): 273 validated Paediatric Investigation Plans (PIPs)
- Committee for Advanced Therapies (CAT): 46 dossiers examined

Consolidation of three Task Forces involving a total of 34 patient representatives and volunteers in the areas of Orphan Drugs; Paediatric Drugs and Drug Information & Transparency & Access; and organisation of one Workshop for each Task Force.

Creation of a Therapeutic Action Group (TAG) in January 2009, composed of all EURORDIS representatives in the above-mentioned scientific committees and working party at the EMA.

Production and dissemination of a monthly report of activities of EMA Committees and EURORDIS' patients' representatives in these Committees.

## Summer School and Drug Development Pedagogic kit

Organisation of the second EURORDIS Summer School in June 2009 in Barcelona. The aim of this 4-day capacity building programme is to train rare disease patient representatives in the areas of drug development, clinical trials and aspects of the EU regulatory process. (39 fellows attended).

E-learning tools were developed and customised for online accessibility, in a downloadable, translatable form and uploaded on the EURORDIS website.

## Charter for Clinical Trials in Rare Diseases

The general aim of the EURORDIS Charter for Clinical Trials in Rare Diseases is to regulate the relationship between a clinical trial promoter and the patient organisations representing the disease concerned by the study.

Launching of the implementation phase of the Charter by:

- Inviting pharmaceutical companies developing medicinal products for rare diseases to formally adhere to the Charter
- Starting the implementation of the Charter with a first case of clinical development in a rare indication. The trial promoter and almost all patient organisations concerned by the trial have been put in contact and are expected to prepare and sign in 2010 an official agreement clarifying the areas of their collaboration.

## Drug Information Association Patient Fellowship Programme

Foster patient representation at the Drug Information Association (DIA) 21st annual EuroMeeting in Berlin from 23-25 March, 2009. For the fourth consecutive year, the DIA Patient Fellowship programme, enabled 33 patient representatives from 13 different countries to participate in this important conference. A special booth dedicated to the patient fellows was set up to increase their visibility.

## Health Technology Assessment Training

Patient representatives were identified and selected to participate in a training session on Health Technology Assessment at the DIA HTA Forum, held on November 25 and 26, 2009 in Paris.

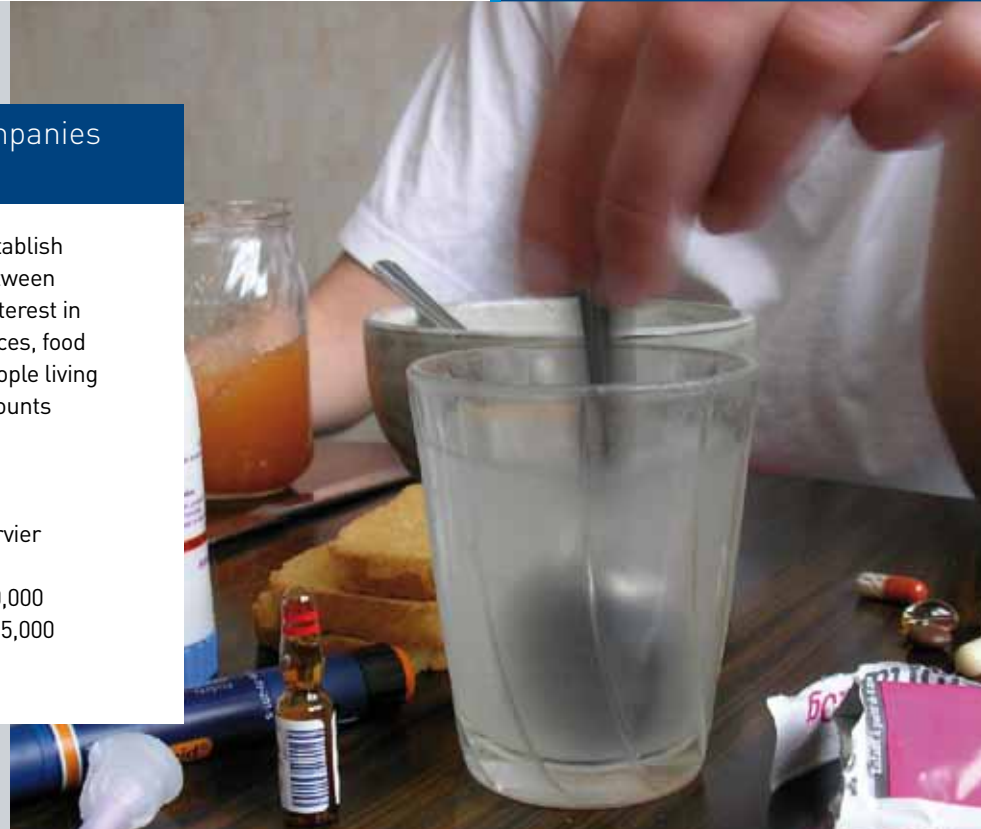
## Collaboration with the French National Institute for Medical Research

Close collaboration with the French National Institute for Medical Research (INSERM) in the area of capacity building for patient representatives in France. The new 2009 training sessions focused on "Clinical Trials in the Paediatric Population".

## EURORDIS Round Table of Companies (ERTC)

Development of the ERTC mission to establish a long term educational relationship between EURORDIS and companies having an interest in orphan drugs, treatments, medical devices, food supplements and health services for people living with rare diseases. In 2009, the ERTC counts with:

- 33 members
- 2 new members (Les Laboratoires Servier and Talecris Biotherapeutics)
- 2 upgrades (Pfizer from € 5,000 to €10,000 and Celgene International SARL from € 5,000 to €25,000)



## EURORDIS Round Table of Companies – 2009 Workshops

### “EURORDIS Strategy 2010-2015 and Partnership with the ERTC Members”

→ 19 June 2009, Barcelona: 50 attendees from 11 countries

To mark the 10th anniversary of the EURORDIS Round Table of Companies (ERTC) Workshops, this meeting focussed on the EURORDIS' vision for the future, a vision shared by the National Organization for Rare Disorders (NORD) with whom a close collaboration is being implemented to coordinate efforts at a global level. This strategy will benefit the development of orphan drugs and will have a direct positive impact on the lives of rare disease patients, in the two continents and beyond. This workshop was also the opportunity to take stock of five years of fruitful partnership with the pharmaceutical companies supporting EURORDIS via the ERTC.

### “Improving Access to Orphan Drugs for all Patients Affected by Rare Diseases in Europe: EU Assessment of Clinical Added-Value of Orphan Drugs” (CAVOD)

→ 11 December 2009, Paris: 63 attendees from 12 countries

Following the outcomes of the EU Pharmaceutical Forum in 2008, the Commission Communication on Rare Diseases (November 2008) and the EU Council Recommendations on Rare Diseases (June 2009), this meeting provided the opportunity for all stakeholders to discuss both the EURORDIS and the EBE(EFPIA)/EuropaBio proposals on how to move towards the practical implementation of an effective and realistic procedure for the centralised evaluation of the clinical added-value of newly authorised orphan medicinal products.



## COMMUNITY BUILDING

EURORDIS brings the rare disease community together and seeks to empower rare disease patients by enabling them to share and learn from each other. We organise structures where the rare disease community can grow and thrive. We believe that our strength is in numbers and in acting together in a coordinated way.



# Information & Networking

## Membership base - 406 members

50 new members joined in 2009 (33 full members and 17 associate members). New countries represented: Latvia, Turkey and the Russian Federation. At the end of 2009, EURORDIS has 406 members in 42 countries, out of which 29 European countries and 25 from the European Union.

## Membership Meeting – Athens 2009

Organisation of one conference entitled “**National Strategies for Rare Diseases: No policy without patients!**”, held in Athens on May 8-9, hosted by the Greek Alliance for Rare Diseases.

Participation of 196 people from 27 countries

## Council of National Alliances

Consolidation of a Network of 20 National Alliances for Rare Diseases.

Two Workshops of the Council of National Alliances focusing on the organisation of the Rare Disease Days 2009 and 2010 and on the development of National Plans for Rare Diseases in the framework of the EuroPlan project.

Support to the creation of a National Alliance for Rare Diseases in Switzerland, launched in February 2010.

## Council of European Federations

Creation of a European Network of 30 European Rare Disease Federations in March 2009.

Two European Workshops of the Council focusing on: building the capacity of the European Disease Specific Networks and promoting best practices for their development (“Rare! Together” programme); collaboration with the European Reference Networks of Centres of Expertise; sustainability of the European Federations and organisation of Rare Disease Day 2010.

## Online patient communities –new ways of connecting

Hosting and maintenance of 28 online patient communities / mailing lists with 951 registered users. The most active mailing lists are for Fragile X, Prader Willi, Sanfilippo, Aicardi and Behçet Syndromes.

Development of a web tool ([www.rarediseasecommunities.org](http://www.rarediseasecommunities.org)). The web tool is a multilingual enabled online-social network for patients and families allowing them to connect with one and other to support and share vital experiences on aspects of living with a rare disease. The project is a joint collaboration between EURORDIS and NORD.

Release of the web tool with one active Online Patient Community for rare auto-inflammatory diseases Cryopyrin Associated Periodic Syndromes (CAPS). ➤

- ▶ A Mentoring Workshop on the use of Social Media Communication was organised at the Membership Meeting of May in Athens. Other meetings were called to recruit moderators and patients in charge of Online Patient Communities in Montpellier and Carquefou (France).

## Informing & raising awareness

Positive change for people living with rare diseases cannot happen if decision-makers, health professionals, researchers and the general public are not aware of rare diseases and what they mean. EURORDIS uses its pivotal position in the rare disease community to inform, educate and raise awareness about rare diseases.

## Website – new design, new functions, new sections

Overhaul of the main EURORDIS website ([www.eurordis.org](http://www.eurordis.org)) available in 6 languages, in order to give it a new look and feel and in order to improve its usability and interface with existing internal databases containing information pertaining to patient groups across Europe. The new website includes new functions to encourage patients to share their testimonies of living with rare diseases via EURORDIS' Facebook Group, Twitter and YouTube Channel. The new website includes an RSS news feed to track EURORDIS' work in the news.

- Number of website visitors/month: 19 800
- Number of websites linking to EURORDIS website: 7 963

## Rare Disease Day 2009 – “Patient Care: A Public Affair!”

Coordination of the Rare Disease Day campaign on and around February 28th, 2009. The campaign was implemented in 30 countries, of which 23 European countries. Various awareness-raising events were organised by EURORDIS and its 19 member National Alliances, together with more 600 patient organisations throughout 5 continents. Events organised in each participating country are visible on: [www.rarediseaseday.org/2009/](http://www.rarediseaseday.org/2009/)

## Electronic Newsletter – 20% more subscribers

Production of 10 issues of the E-Newsletter in 6 languages (English, French, German, Italian, Spanish and Portuguese). Each issue includes 3 to 4 articles, announcements, orphan drug designations and an events calendar. Subscription rate went up 20% in 2009, with 7,171 subscribers from around the world.

## Video Contest – a new source of patient testimonies

EURORDIS' First Video Contest attracted 49 entries from more than 25 different countries, and showcased more than 30 different rare diseases. Most videos are compelling testimonies from people living with rare diseases. All of the videos submitted to the contest can be found on EURORDIS' Rare Disease Day YouTube Channel <http://www.youtube.com/rarediseaseday>

## Blog – a new international forum to advocate for rare diseases

Launching of Rare Disease Blogs ([www.rarediseaseblogs.net](http://www.rarediseaseblogs.net)) an advocacy blog featuring commentary about rare disease policy and orphan drugs from opinion leaders of the international rare disease community. This project is conducted in partnership with the National Organization for Rare Disorders (NORD).

## Press releases – more active external communication

More pro active communication with members, other rare disease stakeholders and the media via e-mailings and press releases. In total, 6 press releases were sent out on: Rare Disease Day; Rare Disease Day events at the European Commission and the European Parliament; the Book “The Voice of 12.000 Patients”; the adoption of the Council Recommendation on Rare Diseases; Patient nominations at the European Medicines Agency's Committee for Orphan Medicinal Products; the Strategic Partnership between EURORDIS and the National Organization for Rare Disorders (NORD). In 2009, EURORDIS hired an on-line media monitoring service to track media coverage of our work.



## INFORMATION SERVICES TO PATIENTS

EURORDIS promotes the implementation of services adapted to the situation and special needs of people living with rare diseases. We facilitate the networking of rare disease Help Lines across Europe and provide patient-friendly access to information through our web sites.



### European Network of Rare Disease Help Lines

Organisation of a Management Meeting in Athens, the day before the Annual Membership Meeting, to establish stricter protocols and a more rigorous procedure to join the European Network of Rare Disease Help Lines. (10 participants)

Organisation of a workshop, at the Annual Membership Meeting, aimed at promoting the expansion of the Network. The workshop was open to applicants interested in creating a Help Lines service. (40 participants)

Onsite visits and trainings to 'start-up help lines services' took place in Italy, Romania, Belgium, Spain, Portugal  
Development of the Second Europe-wide Caller Profile Analysis and Isolated Patients lists, in November 2009, with 9 national help lines.

Production of Factsheets for patient advocates on National Help Lines and the European Network of Rare Disease Help Lines.



## Rare Disease Day 2009

The theme of this year was "Patient Care: a Public Affair", promoting the creation of Centres of Expertise in order to improve access to care and quality of care.

An event was organised in Brussels to mark the launch of the book "The Voice of 12,000 Patients", which analyses the data resulting from two surveys on the needs and the expectations of rare disease patients on diagnosis and access to care. The event was hosted by EU Commissioner for Health Androulla Vassiliou.

A Dinner Debate at the European Parliament was organised by EURORDIS and hosted by Antonios Trakatellis MEP, Rapporteur for the European Parliament on the Council Recommendation on a European Action in the field of Rare Diseases.

A dedicated website was developed which served as a key communication tool: [www.rarediseaseday.org/2009](http://www.rarediseaseday.org/2009). The website contained downloadable resources, an Information Pack and other materials accessible to organisers; a section to upload photos and videos; press releases for journalists. 31 country pages, updated by participating patient organisations themselves, focused on the national events and initiatives organised in each country.

Two media monitoring services were hired to track the impact of the Rare Disease Day: paper press from 11 European countries and online press in 17 countries. Over 1300 on-line article and nearly 300 paper press clippings were collected at the end of the campaign.

A dedicated YouTube channel and a Facebook Group were specifically created for the Rare Disease Day.

1000 posters were produced and disseminated to EURORDIS membership and key stakeholders.



## REVIEW OF EURORDIS' STRATEGY FOR 2010-2015

From September 2008 to November 2009, the Chief Executive Officer with the Board of Directors and staff team, conducted an in-depth review process towards a five-year strategy 2010-2015. This process included several surveys addressed to EURORDIS' members as well as non members and various stakeholders with whom our association interacts with, such as pharmaceutical companies, academia, the European Commission, the European Medicines Agency, the Food and Drug Administration and the National Institute of Health in the United States.

The revised strategy 2010-2015 was presented at the Annual General Assembly on May 8th, 2009 in Athens and will be formally adopted at the Annual General Assembly on May 13th, 2010 in Krakow. The members acknowledged that the role of EURORDIS is to act as a facilitator and to advocate for the involvement of patient representatives in any research, public health, and social projects as well as in any relevant expert, advisory and decision-making committees. EURORDIS has a responsibility to empower member patient organisations and provide capacity building tools as well as channels to exchange information through its web services. We represent all rare diseases, genetic or not, including rare cancers, in all of Europe. EURORDIS will maintain a high level of advocacy activity to promote rare diseases as a public health priority at EU level, in all Member states as well as internationally. We will:

- Enhance our partnership with National Alliances of Rare Diseases to translate European policy initiatives into national plans or strategies on rare diseases.
- Further develop our common strategy with rare disease-specific European Federations.
- Take the European Network of Rare Diseases Help Lines to a new level along other social media and information services to patients and families.
- Focus our efforts on important areas such as: drug development, information and access, research policy, centres of expertise and European reference networks, gene testing, counselling and screening, as well as quality of life.

# Governance, Funding & Organisation



## Board of Directors

Organisation of 4 meetings of the Board of Directors and 4 meetings of the Board of Officers to address EURORDIS' strategic issues:

- Strategic review and development of Strategy 2010-2015
- Work Plan 2009 and 2010
- Advocacy work for the adoption of the Council Recommendation on Rare Diseases (CRRD), EU Committee of Expert on Rare Diseases (EU CERD), Clinical Added Value of Orphan Drugs (CAVOD)
- Approach to future European Conferences on Rare Diseases

- Strategic Partnership with National Organization for Rare Disorders (NORD)
- Rare Disease Day
- Therapeutic Development Programme and greater involvement and support of volunteers in these activities
- Rare disease research policy
- National Plans on Rare Diseases
- Road Map on Web communications & new Web services
- Funding strategy



## Strategic Partnership: EURORDIS & NORD join forces

Strategic Partnership agreement with the National Organization for Rare Disorders (NORD). This agreement is far reaching, bringing together the two main rare disease patient organisations in Europe and the USA, toward greater convergence in the next five years.

The partnership covers activities such as outreach to rare disease patient organisations and capacity building activities, exchanges and joint actions in advocacy, joining forces in international activities to play a pivotal role in promoting rare diseases as an international public health issue, orphan drug development and policy environment, fund raising.

The most significant activities in 2009 are the implementation of similar methodology for the strategic review and intense dialogue on strategic outcomes, the strong collaboration to promote Rare Disease Day in the USA and internationally, the joint action to develop the new service of On Line Patient Communities and the International Rare Disease Blog.

## Staff Team Organisation

The team comprises 22 people, 19.1 full-time equivalent (FTE) as of December 2009, which is quite stable compared to the 17.1 FTE in 2008. The team is composed of paid staff, two seconded staff from AFM-Téléthon and INSERM one office volunteer, two consultants and one free-lance staff. Most staff is based in the Paris office located on the Rare Disease Platform. EURORDIS' European Public Affairs Director and European Public Affairs Advisor are based in the Brussels office.

- Creation of Web Communications Officer position, held by Denis Costello
- Return of Julia Fitzgerald, Web Content Manager, from sabbatical leave
- Creation of an IT and Support Services Manager position, held by Ludovic Inard
- Recruitment of Administrative Secretary trainee

## Volunteers

- 21 volunteers involved in the European Public Affairs Committee (EPAC)
- 48 volunteers involved in EU committees, task forces, working groups and EU-funded projects as well as in various conferences.
- 14 volunteer translators contributing regularly out of a pool of 74

## Finance and Support Services

- Accounting and monthly financial reporting in a timely manner including cash flow and risk analysis detailed report
- Monthly meeting with managers to update the Budget and the Year-end Financial Forecast
- Operating Grant Steering Committees organised every two months with review of progress on implementation, deliverables and budget
- Quarterly collection of activity indicators
- Office space rented in Brussels since May 2009
- 4th Revision and promotion of EURORDIS Policy on Financial Support by Commercial Companies

## Contract Grants

### New

- Operating Grant for year 2009 ("OPERA"), single beneficiary, DG Health and Consumers, 12 months
- Web2.0 On Line Patient Communities pilot, NORD, 18 months
- Web2.0 Capacity Building Workshop, NOVARTIS, event

### Renewed

- Web2.0, The French Pharmaceutical Companies Association (LeeM), 12 months
- Rare!Together, Medtronic Foundation, 12 months
- Summer School Session 2009, Drug Information Association, event
- e-Newsletter, Medtronic Foundation, 12 months

### On Going

- Advocacy and Core activities, AFM-Téléthon, 36 months
- POLKA main partner, DG Health and Consumers, 36 months
- EuroPlan, associated partner, DG Health and Consumers, 36 months
- nEUroped, associated partner, DG Health and Consumers, 36 months
- Treat-NMD (EuroBioBank), associated partner, DG Research, 60 months

### Submitted

- Operating Grant for year 2010 ("EURORDIS\_FY2010"), single beneficiary, DG Health and Consumers, 12 months





## Volunteers ←

EURORDIS' volunteers base plays an important and active role in our activities and projects. There are two kinds of volunteers:

The EURORDIS European Public Affairs Committee (EPAC) comprising 21 volunteers is dedicated to tackling advocacy issues relevant to rare diseases, to determine EURORDIS' position based on consultations with our members, and to represent EURORDIS in EU committees and/or European and international conferences. There are also EURORDIS' volunteers sitting in the various committees of the European Medicines Agency (two in the Committee for Orphan Medicinal Products, two in the Committee for Paediatric Drugs, one in the Committee for Advanced Therapies and one in the Patients & Consumers Working Party). They are the voice of the patients and their work is quite remarkable. They are supported by other volunteers involved in the following task forces: Orphan Drugs, Paediatric Drugs, Drugs, Information, Transparency and Access (DITA). The cumulated number of days of these 21 volunteers accounts for 693 days in 2009.

In addition, 48 other volunteers are also actively involved in EURORDIS' European issues including advocacy, health policy, drug development and research. These volunteers are mostly patients or parents of patients. They are experts of their disease and are fully aware of the common challenges faced by sufferers across Europe. They have extensive experience in patient organisations, are fluent in English and often speak several other languages; they bring to EURORDIS diversity of cultures from different European countries and they cover a large variety of rare diseases.

The cumulated number of days of these volunteers accounts for 174 days in 2009.

The volunteer translators kindly contribute their time to translate EURORDIS' documents in 6 languages: English, French, German, Italian, Portuguese and Spanish. The communication in 6 languages is very valuable to reach patients and their families on an international scale and further consolidates the pan-European community of people living with rare diseases. This extended communication would not be possible without volunteer translators. Our aim is to expand the number of translations into other European languages.

Each person volunteering his or her time to EURORDIS brings a valuable contribution. The work performed constitutes a chain where everyone supports each other.

The Board of Directors comprises 12 directors, who are all rare disease patient group representatives mandated by their organisations; the majority of them are patients or are parents of people affected by a rare disease.



## Boards of Directors

### PRESIDENT

Mr. Terkel Andersen \*

- President

The Danish Haemophilia Association  
Denmark

### DIRECTORS

Ms Avril Daly

Genetic & Rare Disorders Organisation  
Ireland

Ms Dorica Dan

Romanian Prader Willi Association  
Romania

Mr. Jean Elie \*

- Treasurer

Vaincre la Mucoviscidose  
France

Mr. Torben Grønnebak

Rare Disorders Denmark  
Denmark

Mr. Flavio Minelli \*

- Officer

Unione Italiana Ittiosi  
Italy

Ms Béatrice de Montleau

Associations Française contre les Myopathies  
France

Dr. Mirando Mrsic

The Croatian Society of Patients with Rare Diseases  
Croatia

Ms Christel Nourissier \*

- General Secretary

Prader Willi France  
France

Mr Anders Olauson

Agrenska  
Sweden

Ms Rosa Sánchez de Vega\*

- Vice-President

FEDER – Federación Española de Enfermedades Raras  
Spain

Mrs Paola Zotti

UNIAMO – Federazione Italiana Malattie Rare  
Italy

\*Member of the Board of Officers

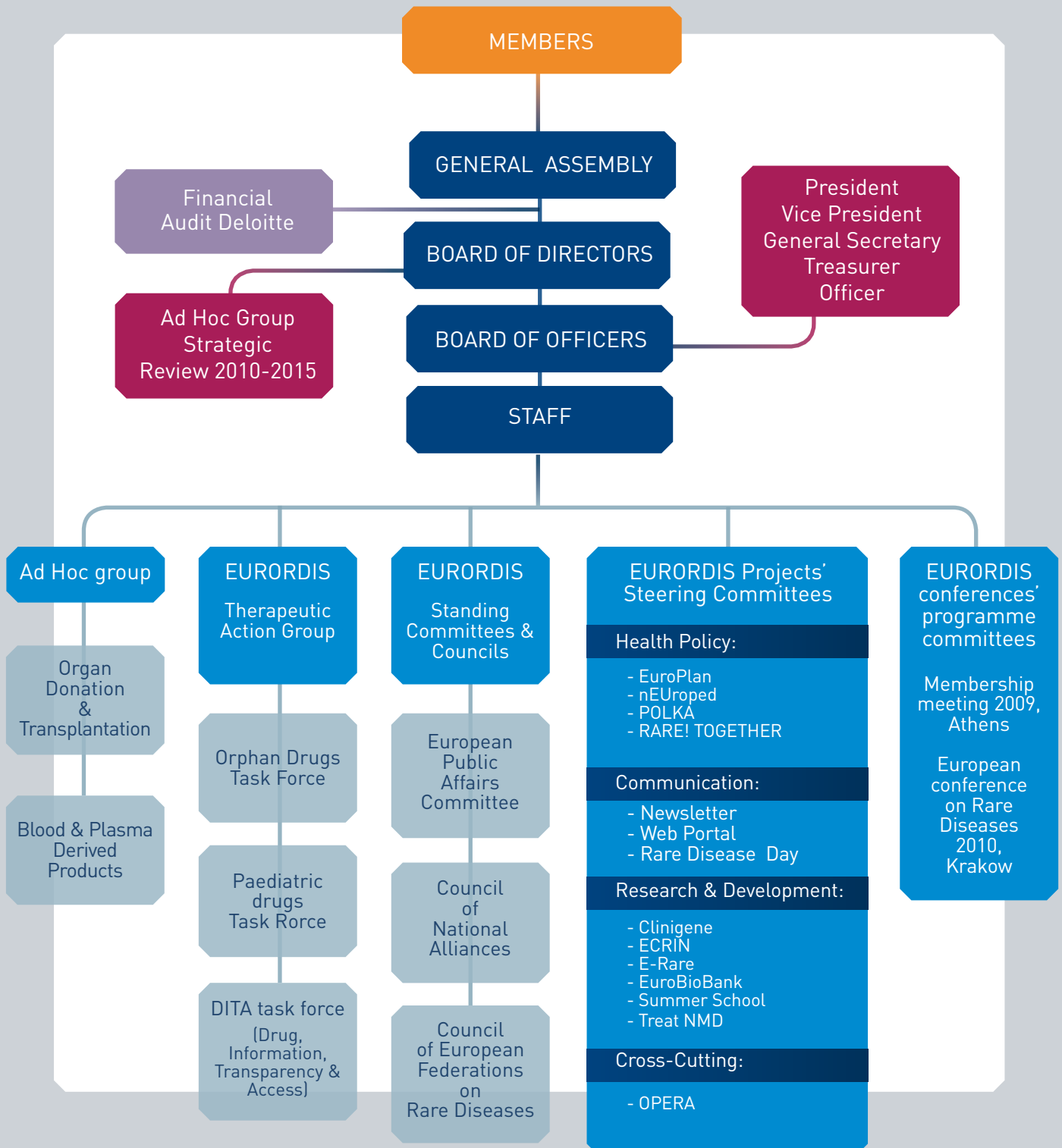
# Members of Eurordis



Argentina	<ul style="list-style-type: none"> <li>• FUNDACION GEISER - GRUPO DE ENLACE, INVESTIGACION Y SOPORTE ENFERMEDADES RARAS</li> </ul>
Armenia	<ul style="list-style-type: none"> <li>• DOCTORS AND CHILDREN HEALTH CARE</li> <li>• NEUROHEREDITARY DISEASES CHARITY ASSOCIATION</li> </ul>
Austria	<ul style="list-style-type: none"> <li>• DEBRA INTERNATIONAL</li> <li>• EUROPEAN HAEMOPHILIA CONSORTIUM</li> <li>• PULMONARY HYPERTENSION ASSOCIATION EUROPE (PHA EUROPE)</li> <li>• SELBSTHILFEGRUPPE LUNGENHOCHDRUCK - AUSTRIAN PH PATIENT GROUP</li> </ul>
Belgium	<ul style="list-style-type: none"> <li>• ASSOCIATION BELGE DU SYNDROME DE MARFAN ASBL</li> <li>• ASSOCIATION DE PATIENTS SOUFFRANT D'HYPERTENSION ARTÉRIELLE PULMONAIRE EN BELGIQUE</li> <li>• ASSOCIATION DE SOUTIEN ET D'AIDE AUX FAMILLES CONCERNÉES PAR LE SYNDROME DE MICRODÉLÉTION 22Q11.2</li> <li>• ASSOCIATION SPINA BIFIDA BELGE FRANCOPHONE</li> <li>• BELGISCHE ORGANISATIE VOOR KINDEREN EN VOLWASSENEN MET EEN STOFWISSELINGSZIEKTE</li> <li>• CONTACTGROEP MYELOOM PATIENTEN</li> <li>• DEBRA BELGIUM vzw</li> <li>• EURO ATAXIA - EUROPEAN FEDERATION OF HEREDITARY ATAXIAS</li> <li>• EUROPEAN MYELOMA PLATFORM</li> <li>• EUROPEAN NETWORK FOR RESEARCH ON ALTERNATING HEMIPLEGIA</li> <li>• EUROPEAN POLIO UNION</li> <li>• FEDERATION OF EUROPEAN SCLERODERMA ASSOCIATIONS</li> <li>• FEWS - FEDERATION OF EUROPEAN WILLIAMS SYNDROME</li> <li>• GROUPE D'ENTRAIDE BELGE DU SYNDROME GILLES DE LA TOURETTE</li> <li>• HAE BELGIUM</li> <li>• ICHTHYOSE BELGIQUE - ICHTHYOSIS BELGIË</li> <li>• RARE DISEASE ORGANISATION BELGIUM</li> <li>• RARE DISORDERS BELGIUM</li> <li>• SIOP EUROPE</li> <li>• VLAAMSE VERENIGING NEUROMUSCULAIRE AANDOENINGEN VZW (NEMA)</li> </ul>
Bulgaria	<ul style="list-style-type: none"> <li>• BULGARIAN ANT THALASSAEMIC ASSOCIATION</li> <li>• INFORMATION CENTRE FOR RARE DISEASES AND ORPHAN DRUGS - BULGARIAN ASSOCIATION FOR PROMOTION OF EDUCATION AND SCIENCE</li> <li>• NATIONAL ALLIANCE OF PEOPLE WITH RARE DISEASES (NAPRD)</li> </ul>
Burkina Faso	<ul style="list-style-type: none"> <li>• FONDATION INTERNATIONALE TIERNO ET MARIAM</li> </ul>
Canada	<ul style="list-style-type: none"> <li>• CANADIAN ORGANIZATION FOR RARE DISORDERS</li> </ul>
Croatia	<ul style="list-style-type: none"> <li>• DEBRA, DRUSTVO OBOLJELIH OD BULOZNE EPIDERMOLIZE</li> <li>• MYASTHENIA GRAVIS SOCIETY OF CROATIA - DRUSTVO OBOLJELIH OD MIASTENJIJE GRAVIS HRVATSKE</li> <li>• THE CROATIAN SOCIETY OF PATIENTS WITH RARE DISEASES</li> </ul>





# EURORDIS Governance chart

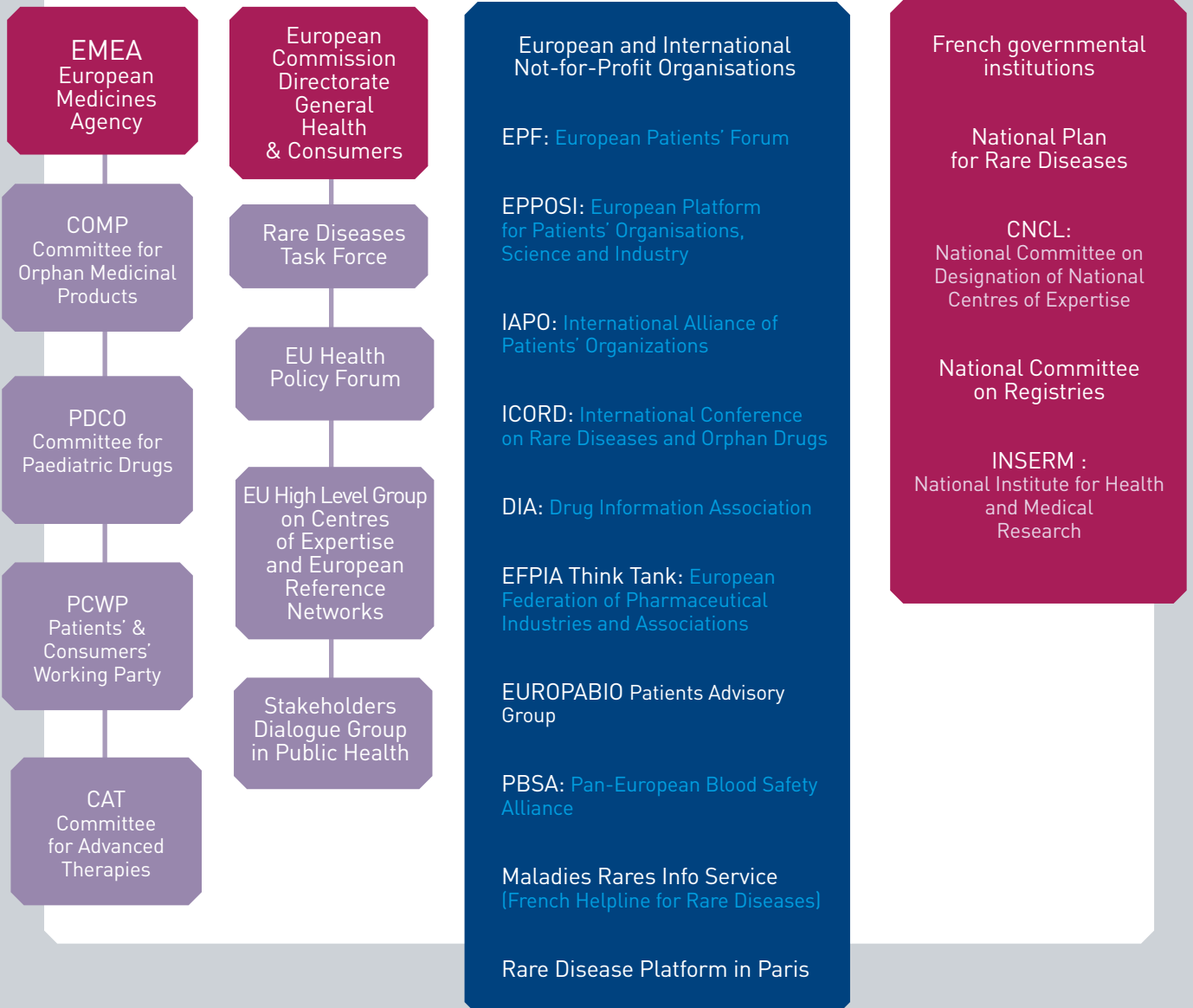




# EURORDIS' representation in external institutions and organisations in 2009

→ Governmental Institutions 

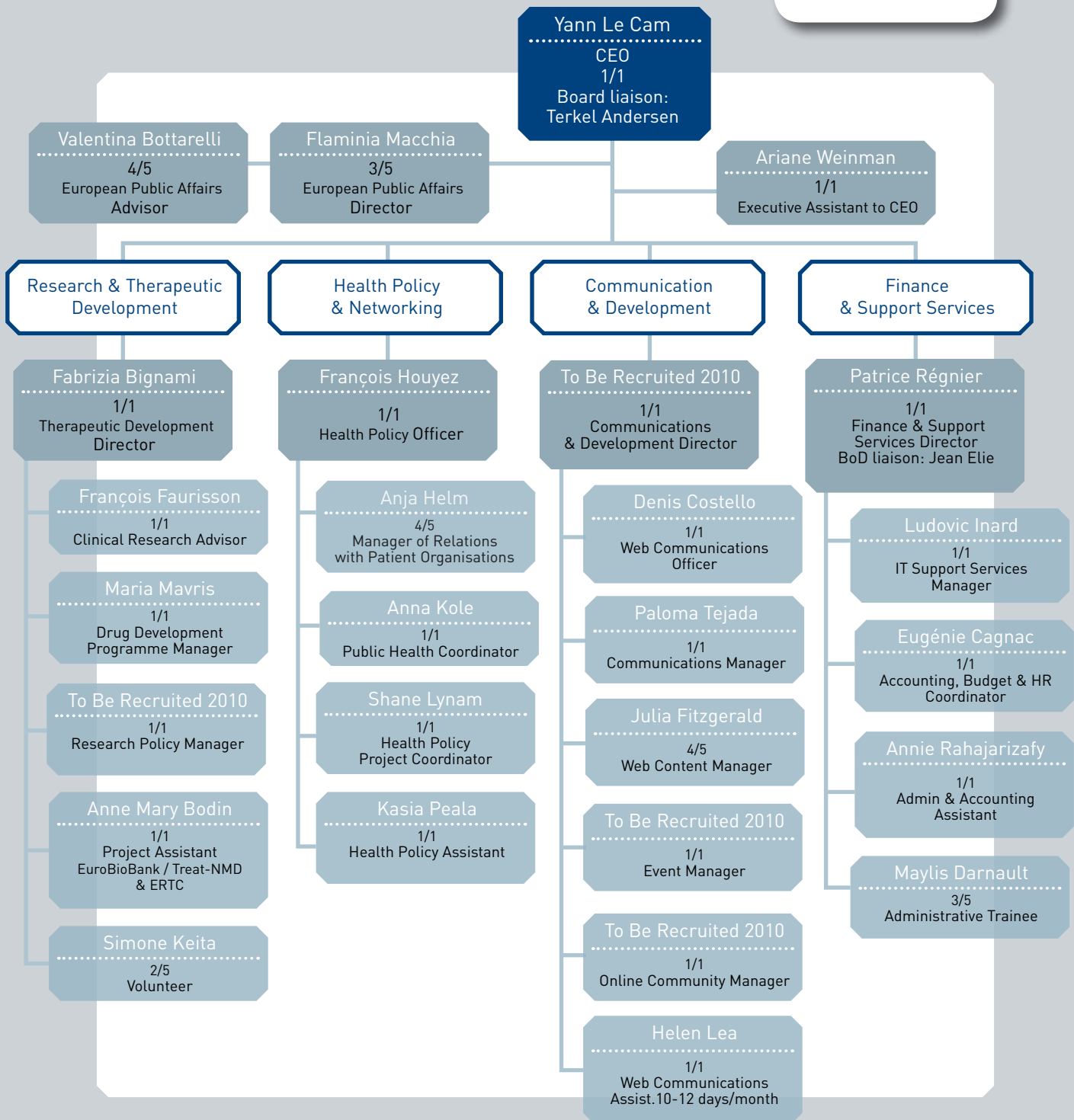
→ Non-Governmental Organisations 



# Organisational Chart 2009



**EURORDIS**  
Rare Diseases Europe

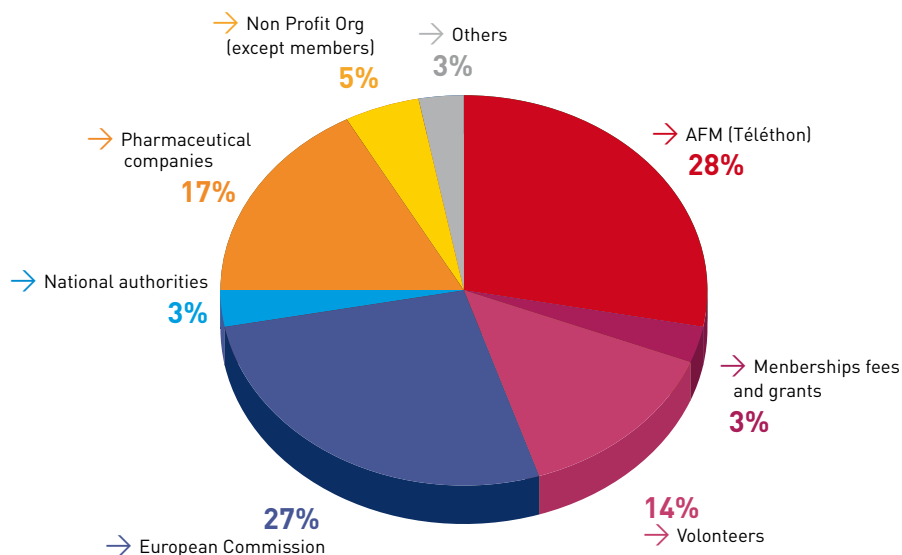


# Revenue and Expenses 2009

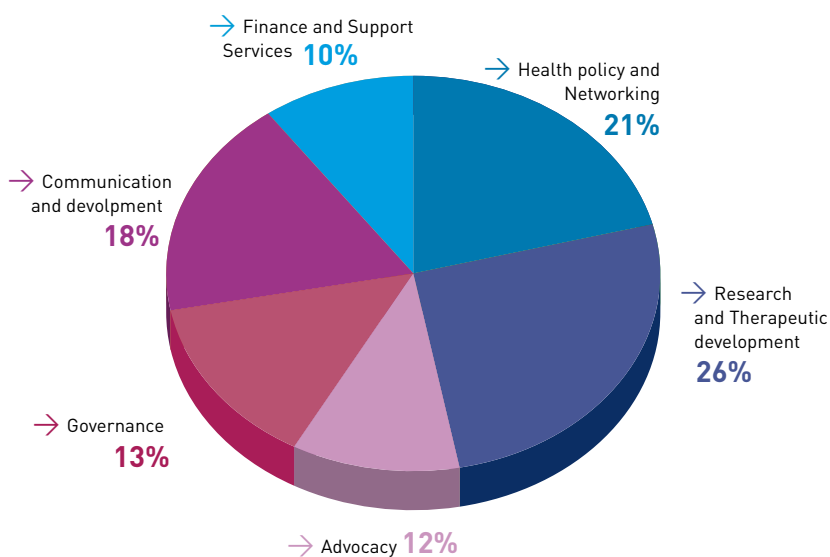
(in thousand of euros, provisions excluded)

3%

→ Revenue 2009  
= 2,862



→ Expenses 2009  
= 2,733





## Participation of EURORDIS' representatives in conferences in 2009

- ➔ Master santé publique, programme d'éducation thérapeutique du malade, Paris 13, Bobigny, France, December 18
  - François Houÿez : « Place des malades dans les politiques de santé européennes »
- ➔ RELEVANT HEALTH INFORMATION FOR PATIENTS & CONSUMERS: A public meeting hosted and chaired by Dr. Thomas Ulmer (Germany, EPP) and Carl Schlyter MEP (Sweden, Greens), European Parliament, Brussels, Belgium, December 2
  - Flaminia Macchia represented EURORDIS
- ➔ PATIENT PARTNER – Central - Eastern European Regional Workshop : Defining the needs and means for more Partnership between Patients, Patient Organisations and Other Stakeholders in Clinical Trials, Budapest, Hungary, November 30 & December 1
  - Gabor Pogany, Beata Boncz and Eva Tabai represented EURORDIS - Gabor Pogany presented the EURORDIS Charter on Clinical Trials
- ➔ DIA Health Technology Assessment (HTA) Forum 2009, Paris, France, November 25 and 26
  - Yann Le Cam: Session Chair of "European Co-operation in HTA – How far can we realistically go?"
  - Michele Lipucci di Paola, Session Chair of "Investigations of the gap between Clinical Added Value and HTA"
  - Lesley Greene: "Can Transparency Help Patients Understand Decisions?" (presentation in the session on: "Will transparency lead to predictability?")
  - Fabrizia Bignami and Maria Mavris attended the DIA HTA Forum
- ➔ Informal workshop: "Universal access to orphan medicinal product, dream or reality?", organised by EC DG Enterprise (Georgette Lalis), November 24
  - Yann Le Cam represented EURORDIS
- ➔ CBI's Inaugural Forum on Compassionate Use Programs "Ensure Global Compliance for Named Patient, Specialized and Expanded Access Programs", London, U.K, November 17-18
  - François Houÿez spoke in the Session: Establish Effective Processes for Developing and Managing Compassionate Use Programs: Key note address on Patients' Views
- ➔ Follow-up meeting after the conference "Assessing Drug Effectiveness", Permanent Representation of Sweden to the EU, Brussels, Belgium, November 13
  - Yann Le Cam represented EURORDIS
- ➔ CONGRESSO NAZIONALE: La sicurezza e la qualità nei trapianti, Florence, Italy, November 13-14
  - Michele Lipucci di Paola: Sessione: "La Medicina Rigenerativa: "Bisogni e Attese Associazioni di Pazienti"





- Colloque Maladies Rares et Orphelines, EUROBIOMED, Montpellier, France, October 29-30
  - Gérard N'Guyen represented EURORDIS in the Scientific Committee and at the conference
  - Christel Nourissier represented EURORDIS in the Conference
- 10th EPPOSI Workshop on Partnering for Rare Disease Therapy Development: 10 years Orphan Medicinal Products Regulation - Where do we go? Belgian Federal Parliament - Brussels, Belgium, October 26 - 27
  - SESSION 2 – BUILDING ON THE POLICY BASE OF THE LAST 10 YEARS TO ADVANCE POLICY IN THE NEXT 5 YEARS
  - Sessions Moderators : Yann Le Cam, EURORDIS, Giulia Del Brenna, EC, DG Enterprise & Industry
  - Maria Mavris, Flaminia Macchia and Ariane Weinman attended the workshop
- 2nd Workshop on the Development of an EMEA Transparency Policy, London, UK, October 19
  - François Houy ez represented EURORDIS
- PATIENT PARTNER - North-Western European Regional Workshop : Defining the needs and means for more Partnership between Patients, Patient Organisations and Other Stakeholders in Clinical Trials, London, UK, October 12 & 13
  - Richard West represented EURORDIS. He presented the EURORDIS Charter on Clinical Trials together with Greetje Goossens
- 12th European Health Forum Gastein: "Financial Crisis and Health Policy", Gastein, Austria, September 30 to October 3,
  - Christel Nourissier: "Treatment of rare diseases: National and European implications from the patients point of view"
  - Rosa Sanchez de Vega and Fran ois Faurisson also represented EURORDIS at the Forum
- 8th European Paediatric Neurological Society Conference & Rare Disorders Symposium, Harrogate, UK, September 29
  - Anna Kole made a presentation of EURORDIS at the Rare Disorders Symposium
- European Network of Centres of Expertise for Cystic Fibrosis, Lymphangiomyomatosis, and Lung Transplantation - Workshop: "How to Build Expertise Networks in Europe", Frankfurt, Germany, September 23 & 24
  - Anna Kole represented EURORDIS
- 3rd Convegno Regionale del Forum delle Associazioni delle Malattie Rare, Pisa, Italy, September 19
  - Terkel Andersen and Michele Lipucci di Paola: "Rafforzamento e Coordinamento a Livello Europeo dei Gruppi di Pazienti con Malattie Rare"
- EUROPLAN Workshop of Work Package 7: "Recommendations for the development of RD national strategies/ plans" ; Amsterdam, The Netherlands, September 17-18
  - Terkel Andersen, Christel Nourissier and Valentina Bottarelli represented EURORDIS and provided inputs to the recommendations
- BBMRI Stakeholder's Forum: "BUILDING A BIOBANKING RESEARCH INFRASTRUCTURE FOR EUROPE", Brussels, Belgium, September 16
  - Fabrizia Bignami: "Biobanks: Patients' Role and Expectations"
  - Valentina Bottarelli also represented EURORDIS
- EB (Epidermolysis Bullosa)2009 Conference, Vienna, Austria, September 6-8
  - Denis Costello: " A Unified EB Patient Database from Diverse Data Sources: It Can Be Done!"
- Swedish EU Presidency conference: "Assessing Drug Effectiveness – Common Opportunities and Challenges for Europe", Stockholm, Sweden, July 28–29
  - Yann Le Cam, panelist in the workshop on orphan drugs
- UNIAMO 10th Anniversary: "1999-2009: Ten years of Federal Associations for Rare Diseases", San Servolo Island -Venice, Italy, July 2-5
  - Yann Le Cam: "The European Context and the European Rare Diseases Patients' Perspective"

- ➔ E-Rare Workshop: "The Future Themes and Needs of Rare Disease Research Funding: What are the Bottlenecks?", Brussels, July 1,
  - [Fabrizia Bignami and Yann Le Cam: "Needs to Boost Research on Rare Diseases: the Patients' Point of View"](#)
- ➔ 7th International Conference for Organizations Representing People with CLM or GIST, "New Horizons in Treating Cancer", Lisbon, Portugal, June 26- 28
  - [Denis Costello, speaker in the session "Web Communication, Social Media and Patient Networking"](#)
- ➔ BALKAN CONGRESS FOR RARE DISEASES, Cluj- Napoca, Romania, , June 26-27,
  - [Christel Nourissier and Dorica Dan were part of the Programme Committee](#)
  - [Christel Nourissier:](#)
    - ["National Policies for rare diseases in Balkan area: The Framework"](#)
    - ["EU Initiatives to Improve the Access to Care for Rare Disease Patients"](#)
    - ["EURORDIS: Focus on Social Services"](#)
    - ["Rare Disease Communities, a EURORDIS project: Toward an online patient community portal for rare diseases"](#)
- ➔ Patients-Professional meeting "Porte Aperte" (open doors) at the end of the 84° Congress of the Dermatologist's Italian Society, Florence, Italy, June 13
  - [Flavio Minelli: "Malattie Rare in Europa: L'azione dei pazienti": Presentation of EURORDIS](#)
- ➔ EULAR CONGRESS 2009 (The European League Against Rheumatism), Copenhagen, Denmark, June 13
 

[Terkel Andersen: Session: "Rare but not less severe - the challenge to care for people with rare rheumatic diseases," : Presentation of EURORDIS](#)
- ➔ 4th Eastern European Conference for Rare Diseases and Orphan Drugs: "Together for Integrative Approach to Rare Diseases", Plovdiv, Bulgaria, June 13-14
  - [Christel Nourissier: Session 1 on "Policy on rare diseases": "EURORDIS: Focus on Social Services"](#)
  - [Dorica Dan: Session 2: "Sharing best practices on integrative approach on rare diseases in different countries": RONARD – STRATEGIES FOR DEVELOPMENT"](#)
- ➔ 1st European Congress on Rett Syndrome: "From research to treatment: New perspectives in Rett Syndrome", Milan, Italy, June 5-7
  - [G rard N'Guyen represented EURORDIS and was part of the Scientific Committee](#)
- ➔ Czech EU Presidency conference: "Treatment of Rare Diseases and EU legislation", under the auspices of the Committee on Health Care of the Chamber of Deputies of the Parliament of the Czech Republic, Prague, May 21
  - [Yann Le Cam: "Improving Access to Orphan Drugs: From EU agreed principles and policies to concrete measures in national strategies on rare diseases".](#)
- ➔ «Quelles conditions pour favoriser l'acc s au march  des m dicaments», debate chaired by Jean-Fran ois Lemoine, LEEM, Paris, France, April 28
 

With Eric Abadie, Laurent Degos, Nicolas About, Christian Iajoux (LEEM President)

  - [Yann Le Cam: « Acc s des patients aux m dicaments orphelins : Des principes politiques valid s aux bonnes pratiques - Le cas de la France en Europe »](#)
- ➔ "Rare diseases and neonatal screening: Views of parents and patients", workshop under the auspices of the Czech EU Presidency.
 

(This workshop was integrated within «The 6th ISNS European Regional Meeting in Neonatal Screening» conference); Prague, Czech Republic, April 26

[Christel Nourissier: "Eurordis action for rare diseases at EU level : building of consensus on neonatal screening"](#)

Additional: Presentation of the PKU Romanian president





- EPPOSI workshop on clinical trials: "Shaping the future of European Clinical Research Legislation", Brussels, Belgium, April 22
  - Gerard Nguyen: "How to measure quality in CTs ?Patients' and PCO's point of view".
- Symposium on Rare Diseases & Dutch Health Policy (organized by VSOP), The Hague, Netherlands, April 8
  - Yann Le Cam: "The European Context and the European Rare Diseases Patients' Perspective"
- CliniGene Annual Meeting, Annecy, France, April 2-5
  - Michele Lipucci di Paola represented Eurordis
- 1ère Rencontre des Associations Suisses de Maladies Rares, Geneva, Switzerland, March 28
  - Christel Nourissier: « L'organisation européenne pour les maladies rares »
- Informal workshop meeting on orphan medicines, European Commission, Brussels, March 27
  - Participation of Yann Le Cam
- EU Pharmaceutical Forum: Delivering for Patients: How to move from agreed principles to good practice and positive change across Europe?, European Commission, Brussels, Belgium, March 25
  - Yann Le Cam: 'Improving Access to Orphan Drugs: From agreed principles to good practices - Case Study: France & EU Pharma Forum'
- DIA EuroMeeting 2009, Berlin, Germany, March 23 - 25
  - Yann Le Cam was the co-theme leader of the theme "Media, Society and Research"
  - Yann Le Cam, Chair of the session "Medicines are about more than money. Building social values into health economics".
  - François Houÿez, panelist in the session "communicating benefit / risk to the public: 12 years after the Erice Declaration, how much progress has been made?"
- Clinigene Conference: "Regulating Gene Transfer Clinical Trials. A Patients group driven initiative: Towards an appropriate and proportionate regulatory framework for gene transfer clinical trials", Abbaye de Royaumont, France, March 6 & 7
  - Michele Lipucci di Paola represented Eurordis
- COMP Meeting, EMEA, London, March 4
  - Yann Le Cam: "Why and how to communicate on orphan designation review at the time of marketing authorization"
- «Focus on Rare Diseases in Ireland; What is the National Plan?», organised by IPPPOPI, the Genetic and Rare Disorders Organisation (GRDO) and the Medical Research Charities Group (MRCG) within the World Rare Disease Day. European Public Information Centre (EPIC), European Union House, Dublin, Ireland, February 25
  - Christel Nourissier: "Why and how to communicate on orphan designation review at the time of marketing authorization"
- Morbus Gaucher - Example for a rare disease - Meet the European Patient Group (Organised by the European Gaucher Alliance and Dr. Peter Liese, MEP) – European Parliament, Brussels, Belgium, February 10
  - Flaminia Macchia represented Eurordis
- 12° CONVEGNO Patologia immune e malattie orfane 2009, Torino, Italy, January 22-24
  - Michele Lipucci di Paola: "L'esperienza di Eurordis"

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- The European Project for Rare Diseases National Plans Development (EUROPLAN)
- The Operating Grant for Rare Disease Associations (OPERA)
- The European Network on Rare Paediatric Neurological Diseases (nEUroped)

## → EUROBIOBANK



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## → ROUND TABLE OF COMPANIES

EURORDIS is grateful to the organisations listed below for their financial support through the EURORDIS Round Table of Companies

- Ruby Members



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**EURORDIS**  
Rare Diseases Europe

Comité éditorial  
Eurordis

Conception et réalisation

Agence Galilée, tél. : 01 43 63 74 04, [www.agencegalilee.com](http://www.agencegalilee.com)





# EURORDIS

Rare Diseases Europe

## EURORDIS

Plateforme Maladies Rares  
102, Rue Didot, 75014 Paris - France

Tel + 33 1 56 53 52 10

Fax + 33 1 56 53 52 15

Brussels office: Tel/fax + 32 2 733 81 10

EURORDIS@EURORDIS.org