ACTIVITY REPORT
2007

Paris, March 2008
Eurordis in 2007: Raising political and public awareness successfully

I am increasingly proud of Eurordis as a unique pan-European patient organisation with high standards of governance. Eurordis leads the way in many areas of social innovation and has created effective partnerships in order to shape a better environment for rare diseases.

In 2007 and early 2008 we can already identify important milestones in raising political and public awareness. The European Conference on Rare Diseases 2007 Lisbon was extremely successful as well as the historic score of contributions sent in response to the Public Consultation regarding European action in the field of rare diseases. The first Public Hearing on Rare Diseases at the European Parliament was also a success. In addition, the first Rare Disease Day on the 29th of February, spearheaded by the national rare disease alliances and spreading across Europe and beyond, reflects how effective we can be when articulating our activities at European and national levels.

In 2008 we must remain focused. On our agenda we still have the adoption of the Commission Communication and the Council Recommendations on Rare Diseases leading to National Plans for Rare Diseases in all Member States. Beyond 2008, we will have a stake in the implementation of these new policies and will look forward to proper measures when the EU Directive on Health Services and Patient Mobility will be adopted. We will also follow closely the legislative packages on organ donations and transplantations and on blood and plasma derived products.

If we are successful in 2008, we will have a clear and promising common road map for the next five years.

Terkel Andersen
President
2007 was an important year for a better future for people living with rare diseases in Europe.

We are about to complete a comprehensive EU regulatory framework to translate research advancements into medicines available to patients. The EU Regulation on the Paediatric Use of Medicines was adopted at the beginning of 2007 and its implementation started in July. The EU Regulation for Advanced Therapies (gene and cell therapies and tissue engineering) was adopted by the European Parliament in May and by the Council of Ministers in October and formally published in December 2007. The development of orphan drugs continues to be successful in the EU with over 500 orphan drug designations and 50 orphan drugs authorised.

There is rising awareness of the lack of access to life-saving therapies. The concept of European collaboration, in order to issue common scientific assessment reports on the clinical added value of orphan drugs as a way to speed up the decision-making process, is moving ahead. Now, we will look carefully at the EU Pharmaceutical Forum process as we expect orphan drugs to be selected as a pilot for a genuine EU Single Market from the patient’s perspective.

2007 was a turning point to push rare diseases as the highest priority ever in the EU 7th Framework Programme for Research. The DG Research Conference “Rare Diseases: Building on Success” was a political milestone. Eurordis celebrated its 10th anniversary in May with a successful Membership Meeting 2007 Paris. The meeting gathered 270 participants dedicated to building the capacities of patient groups in order to gain access to rare disease research resources and liaise with scientists to promote European research projects.

Since 2007, rare diseases are a well identified priority in the EU Health Strategy 2008-2013. The European Conference on Rare Diseases 2007 Lisbon was another milestone, with over 400 participants from 35 countries gathered to review national and European policies toward more patient centred rare disease health care. 2006-2007 has also been a turning point in promoting policies for centres of expertise and European Reference Network for rare diseases. Our 12 national workshops and the European workshop in Prague in July, overall bringing together more than 300 participants, enhanced the dialogue between patient representatives, experts and policy makers. These encounters served to address patients’ expectations and to strengthen consensus on key concepts for future common approaches.

Above all, Eurordis is establishing a strong pan-European community. The first evidence is provided by the steadily-increasing number of member organisations: 310 patient groups in 34 countries). In addition, three new national alliances were formed in Romania, Bulgaria and Hungary, and three new European networks for help lines, respite care services and therapeutic recreational programmes. More evidence is provided by the rapidly growing network of 180 volunteers regularly involved in our activities. There has been a stunning increase in rare disease patient representatives’ involvement with over 700 people participating in the capacity building sessions,
trainings, workshops, conferences, surveys and online communities organised by Eurordis.

I extend a warm thank you to all our members, volunteers, academic and corporate partners and to our grant givers for their commitment and trust.

Yann Le Cam
Chief Executive Officer
**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.

It was founded in 1997 by four patient groups from different therapeutic fields: AFM – Association Française contre les Myopathies (French Muscular Dystrophy Association), Vaincre la Mucoviscidose (French Cystic Fibrosis Association), LNCC - Ligue Nationale contre le Cancer (French League against Cancer), and AIDES Federation.

Today, it is supported by its members and by AFM, the European Commission, corporate foundations, and the health industry.

Eurordis is the voice of 25 million patients affected by rare diseases throughout Europe.

### Key figures 2007:

- 310 member organisations (272 full members, 38 associate members)
- 13 national alliances
- 34 countries, among which 23 European Union countries
- More than 600 rare disease patient groups in Europe represented (310 members + more than 300 allied members)
- more than 1,000 rare diseases
- 18 staff, 16 FTE in Paris and Brussels
- 182 volunteers
- 700 patient groups involved in Eurordis' activities
- Eurordis spoke in 44 conferences, congresses and symposia
- Revenue in 2007: 2.626.000 €

### 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 - 2009

Rare Diseases: a priority visible on the European agenda
- Promote rare diseases public health policies at EU and national levels
- Focus on key advocacy issues
- Web communications
- Launch the first Rare Disease Day 2008 and Rare Disease Day 2009 awareness campaign

A broader grassroots patient-centred community
- Bridge the gap with our members
- Reinforce our patient group membership base
- Special focus on new and future Member States
- Develop individual membership
- Involve more volunteers in our activities
- Organise capacity building and training sessions for patient representatives

Budget growth and funding diversification
- Increase EU support to Eurordis and rare disease activities
- Develop private funding and partnerships with corporations and foundations outside the health sector
- Develop individual support and fund raising events

With a special focus on:
- Commission Communication and Council Recommendations
- European Conference on Rare Diseases – 2007 Lisbon, 2010 Cracow
- Public Health activities
  - Centres of Expertise and European Reference Networks
  - Patient mobility
  - Diagnosis and gene testing
- Services to patients and their families
  - Internet patient on-line communities
  - European networks of help lines
  - Isolated patients
  - European networks of therapeutic recreation programmes
  - European networks of respite care centres
  - Integration at school
- Therapy development
  - Orphan drugs
  - Advanced therapies
  - Clinical trials
- Research infrastructure
  - Biobanks
  - Patient registries
  - Clinical research
Networking and information sharing
- More members (+13%) in more countries (+2 new countries)
- More website visitors (+52%) and more newsletter subscribers (+27%)
- More rare disease mailing lists (total 17) and more subscribers (+57%)
- Eurordis 10th Anniversary: Membership Meeting 2007 Paris and Gala Dinner at Eiffel Tower

Advocacy and policy development
- Promotion of European Commission Communication and Council Recommendations on Rare Diseases
- Launched Public Consultation on Rare Diseases at European Rare Disease Conference 2007 Lisbon
- Eurordis contribution to the Public Consultation towards the Commission Communication
- Mobilisation of the rare disease community to contribute to the Consultation: more than 600 contributions, record response to a consultation on public health
- 4th Survey on availability of orphan drugs and advocacy on solutions
- Adoption of European Regulation on Advanced Therapies Medicinal Products
- Position Papers on Patient Mobility and Cross-border care and on Centres of Expertise and European Reference Networks
- Several Position Papers on Information to Patients on Medicinal Products, on Specialised Social Services and on the future of the EU Single Market in Pharmaceuticals for Human Use

Access to information, diagnosis, treatment and care
- European Conference on Rare Diseases Lisbon - November 2007: 403 participants, 35 countries
- Implementation of the third EurordisCare survey on patients’ experience and expectations on healthcare services: 137 patient groups, 16 rare diseases, 23 countries, 15 languages, 6000 responses
- 12 national workshops in the Czech Republic, Denmark, France, Germany, Hungary, Italy, Luxembourg, Netherlands, Portugal, Spain, Sweden and UK + European workshop (Prague) on Centres of Expertise and European Reference Networks
- Creation of online networks of European services for rare disease patients: Help Lines, Therapeutic Recreation Programmes and Respite Care Services.
- Capacity building for rare disease patient organisations in research activities (CAPOIRA Project). Trainings in clinical protocols in Spain, Italy and Denmark

Therapeutic development and research
- Participation in the Committee of Orphan Medical Products (COMP) at the European Medicines Agency (EMEA): 98 orphan drugs designated, 15 marketed in 2007
- Launch of Treat NMD Project and continuation of Eurobiobank Network
- 2 successful workshops of the Eurordis Round Table of Companies (ERTC) and 8 new members (total 33 members)

Funding and organisation
- Adoption of a fundraising and partnership strategy
- Development of a new comprehensive database for contact management
- Improved monthly financial reporting and transparency of financial information
Networking and Information Sharing

Objectives:
- Expand the network
- Support EU enlargement
- Expand outreach
- Enhance volunteers’ role
- Build the capacity of patient organisations

Membership base
- 41 new members have joined Eurordis (24 full members and 17 associate members)
- 2 new countries represented: Cyprus and Slovenia
  At the end of 2007, Eurordis has 310 members in 34 countries, out of which 27 European countries and 23 from the European Union
- Country visits to Belgium, the Czech Republic and Portugal to meet rare disease patient groups, enhance collaboration with Eurordis and encourage national collaboration between patient groups

Membership Meeting 2007 Paris
- Held in Paris on 4-5 May on the occasion of the European workshop “Gaining Access to Rare Disease Research Resources” at the Institut Pasteur in the framework of the CAPOIRA project, supported by the European Commission DG Research
- 265 participants from 27 countries
- One conference entitled “Understanding Rare Disease Research Resources”
- Two workshops:
  - “European tools for rare disease research”
  - “Getting involved in research”

Website
- The number of visits to the site increased from 381,000 in 2006 to 578,000 in 2007 (+52% increase)
- A project to redesign the organisation and enrich the content was completed in 2007
- The pool of volunteer translators grew to over 200 volunteers in more than 12 languages
- Creation of a website for the first Rare Disease Day 2008
Reflection process initiated on Web 2.0 with new tools for rare disease patients (blogs, wikis, on-line social networking) and new functionalities

Newsletter
- Increase in the number of subscribers by 27% (3,800 subscribers worldwide at the end of 2007) and, more importantly, wide circulation among patient groups in Europe

Photo contest
- Successful 2nd Eurordis photo contest (52 participating families have submitted photos and 4 winners were awarded prizes at the Membership Meeting 2007 Paris)
- 3rd Eurordis photo contest launched in December 2007. Winners will be announced at the Membership Meeting 2008 Copenhagen.

Online patient communities
- After pilot phase in 2006, 2007 was the experimentation phase
- 6 new lists launched in 2007 for a total of 17 lists as of December 2007
- 365 new subscribers (total of 636 subscribers) and 2299 messages exchanged

Publications
- Position papers
- Fact Sheets on “What is a Rare Disease?”; “What is an Orphan Drug?”; “Orphan Drugs: The Role played by Eurordis”, “Rare Disease Patient Groups in the European Union”; “Paediatric Drugs and Rare Diseases”; “The Eurordis Round Table of Companies”, “Survey of the Delay in Diagnosis for 8 rare diseases in Europe (Eurordiscare2)”
- Brochures on the Rapsody project and new patient services
- Brochures on National Alliances for Rare Diseases in Europe
- CD Rom proceedings of European Workshop “Gaining Access to Rare Disease Research Resources”, Paris, May 2007 (proceedings and specific recommendations made by patient groups for improving patient group access to rare disease research resources)
- CD Rom proceedings of European Workshop on Centres of Expertise and Reference Networks for Rare Diseases”, Prague, July 2007
- CD Rom proceedings of Eurordis Round Table for Companies VIth Meeting, Barcelona, July 2007, “Do Rare Disease Patients Have Real Access to Orphan Drugs in Europe?”
- CD Rom proceedings of Eurordis Round Table for Companies VIIth Meeting, Paris, December 2007, “Proof of Concept and Level of Evidence in Orphan Drug Development”
Rare Disease Patient Solidarity Project (RAPSODY) 2006-2008
Project funded by the European Commission and launched in May 2006
Reflection process on Centres of Expertise and European Reference Networks, linked to the EurordisCare 3 survey:

- **12 national workshops** in the Czech Republic, Denmark, France, Germany, Hungary, Italy, Luxembourg, Netherlands, Portugal, Spain, Sweden and UK, from March to May 2007
- **European workshop on July 12-13 in Prague**
  - Overall 350 participants, all stakeholders
  - Comprehensive report (CD-ROM)

Creation of **European networks of services for patients**

- **Help lines for rare diseases**
- **Respite care services**
- **Therapeutic recreation programmes**

with the creation of a database on these services, definition documents, best practices and benchmarking activities

- Creation of a common tool for help lines on very isolated patients, and legal advice on the European legislation on data privacy
- Training session for help line respondents in Alicante, Spain, 22 October 2007

**4th European Conference on Rare Diseases Lisbon 2007** “Patients at the Heart of Rare Disease Policy Development”, November 2007, with 403 participants from 35 countries. Successful launch of the consultation on the Proposal for a Commission Communication to the European Parliament, the Council, the Economic and Social Committee and the Committee of the Regions on a European action on Rare Diseases.

- **Partners:** AFM, Barrestown, Children Living with Inherited Metabolic Diseases (CLIMB) UK, FEDER – Federación Española de Enfermedades Raras (Spanish Rare Disease Federation), Norwegian national centre for rare disorders and disabilities (FRAMBU), Fundacio Doctor Robert, Orphanet, Rare Disorders Denmark, Czech Drug Agency (SUKL).
- Project co-funded by the Portuguese Ministry of Health and private partners: Baxter International Foundation, Sigma Tau Pharmaceuticals, Actelion, UGIM
- Interim report to Public Health Executive Agency and partners

**Objectives:**

- Promote rare diseases as a public health issue
- Develop Eurordis’ capacity of contributions
- Access to information
- Access to diagnosis, treatment, and care
Capacity Building for Patient Organisations in Research Activities Project (CAPOIRA) 2006-2008

- Project funded by the European Commission and started in 2006 (18 month duration)
- Partners: European Clinical Research Infrastructures Network (ECRIN) and 3 national rare disease alliances: FEDER in Spain, UNIAMO in Italy and Rare Disorders Denmark
- The project aims to foster the participation of patient organisations in research activities by increasing their knowledge, skills and capabilities in the two areas of clinical trials and EU-funded rare disease research policy and projects
- European Workshop on "Gaining Access to Rare Disease Research Resources", Paris, May 4-5, 2007
- 20 fellowships (travel and accommodation) for patients and patient representatives to take part in the European Workshop
- 5 training sessions on ‘Understanding Clinical Trial Protocols’ in three different countries (Italy, Spain and Denmark)
- Training materials translated into three different European languages and available in English, Italian, Spanish, Danish
- 62 trained patient representatives

EurordisCare 3 (part of the Rapsody project)
- Launch of a new survey on patients’ experience and expectations concerning access to health services for 16 rare diseases in 23 countries in Europe. 137 patient groups are involved in the study and 6000 questionnaires in 15 languages were received. Collaboration with two INSERM – Institut national de la santé et de la recherche médicale (French National Institute for Health and Medical Research) units for Bio Statistics and Public Health. Survey financially supported by the European Commission and two private partners: Actelion and UGIM

DG SANCO Rare Disease Task Force
- Contribution to the Task Force on Rare Diseases of the DG Health and Consumer Protection (DG SANCO), with special emphasis on the Commission Communication on Rare Diseases

Public consultations in the domain of Health Policy
- Contribution to the Commission consultation regarding Community Action on Health Services (Patient Mobility) - January 2007
- Comments on the European Commission’s Draft Report on current practice with regard to the provision of information to patients on medicinal products – June 2007
- Commission Communication on Rare Diseases, with two specific contributions on (a) respite care services, therapeutic recreation programmes and help lines for rare diseases (b) centres of expertise and European Reference Networks for rare diseases
European Medicines Agency (EMEA)

- Contribution to the EMEA activities by fostering participation of patient groups in the agency's processes and improving public transparency
  - **Patient and Consumer Working Party (PCWP):** 2 members (1 full and 1 alternate): work in transparency, drug information, drug monitoring, and risk communication
  - Observer to the Health Care Professionals Working Group at EMEA
  - Reflection process on risk/benefit communication, proposal of a scale for the severity/frequency of adverse drug reactions
  - Participation of patient representatives in Protocol Assistance for orphan drugs
  - Contribution to and validation of patient-friendly Summaries on Orphan Drug Designation (SMOPS)
  - Participation to the review of first European Public Assessment Reports (EPAR) for orphan drugs.
  - Expert opinion on the assessment of the risk/benefit and risk management plan for thalidomide and lenalidomide at EMEA
  - Contribution to the Meduse Project on “Health Security Agencies in Europe: Between Technocracy and Democracy”, Liège, November 2007

Drug Information, Transparency and Access Task Force

- Creation of the new Eurordis Drug Information Transparency Access Task Force: mandate, first meeting on May 3rd 2007 with 11 members
- Five volunteers to participate in QRD activities at EMEA (Quality Review of Documents: package leaflets, European Public Assessment Report summaries, Q&A documents)
- Participation of these volunteers to the EMEA training in February 2007
- Consultation on the Eudrapharm project

Agence Française de Sécurité Sanitaire des Produits de Santé (French Health Products Safety Agency)

- Follow up of activities, participation to the plenary meeting, June and October 2007

Drug Information Association (DIA) EuroMeeting 2007 in Vienna

- Tutorial on “Active involvement of patient representatives in the regulatory process”
- One-day track for patient representatives with the participation of 12 patient representatives as speakers throughout the Meeting. In addition, there were also other topics of interest such as paediatric medicines, drug information to patients, clinical trials, registries, human tissue engineering, cell therapy, gene therapy, etc
- 42 patient representatives fellowships: 20 full fellows (including 17 speakers) and 22 fellows with fee-waivers
A great achievement for European rare disease patients was the adoption by the European Parliament (April 2007) and Council of Ministers (May 2007) of the EU Regulation on Advanced Therapies Medicinal Products, after relentless advocacy activities by Eurordis.

Brussels office

- Continuation of our Brussels office with a European Public Affairs Director and a European Public Affairs Advisor
- to increase our advocacy capacity, developing and disseminating our position papers
- to promote funding opportunities for rare diseases from EU public institutions

Policy development and governance process improvement

- The Eurordis European Public Affairs Committee (EPAC) plays a crucial role in the advocacy activities of Eurordis. It is a permanent committee of Eurordis, whose members have an official mandate to represent the organisation. The EPAC has three main purposes: to share information among Eurordis representatives to ensure they all have the same up-to-date level of information; to discuss views and seek support from other Eurordis colleagues; to define the position of Eurordis on specific issues.
- Wider consultation of members (when possible time-wise and relevant): e.g. patient mobility, centres of reference.
- Development of guidelines for the elaboration of position papers; these guidelines and description of process can be found on Eurordis’ website.

Adaptation of EU Regulation on Advanced therapies

- First 4 months of 2007 were very busy with front line advocacy in the field of Advanced Therapies Medicinal Products, until the adoption of the proposed Regulation – at first reading - by the European Parliament plenary in April 2007 and the adoption – by unanimity – by the Council, in May 2007. The work entailed the production of press releases, two one-page statements and building consensus with other European-wide patient organisations, writing emails to Members of the European Parliament, Commission’s officials, Member States representatives, meetings with journalists and organising Press Conferences.
milestone was Eurordis’ well-balance and well-argued position on research and therapies based on embryonic stem cells.

- Medium priority in work plan 2008

### Pushing up Rare Diseases as a public health priority

- Commission’s Public Consultation “Rare Diseases: Europe’s challenges” (the work started in 2007 and will last until 2009). Participation to the Drafting Group of DG SANCO and the Rare Disease Task Force
- European Conference on Rare Diseases 2007 Lisbon was the launching pad for the Public Consultation
- Core Eurordis Contribution developed through the second half of 2007
- Eurordis mobilised its network to generate qualitative answers
- Rare diseases have been reinforced as a priority in the EU Public Health Programme (2008-2013) and also high priority in the DG SANCO Work Plan 2008
- Highest priority in work plan 2008
- Rare Disease have been reinforced as a priority in the EU 7th Framework Programme for Research and Development (FP7) and a call for proposals has been launched in 2007
- After Summer 2007, work leading to the organisation of the Public Hearing on Rare Diseases in the European Parliament, also through promotion of the concept of Rare Disease Day 2008.

### Promoting centres of expertise, European Reference Networks and patient mobility

- Eurordis response to the EC consultation regarding Community action on health services (end of January 2007)
- Regular interaction with and participation to the EU High Level Group on Health Services and Medical Care Working Group on Centres of Expertise and European Reference Networks
- Specific contribution on Centres of Expertise and European Reference Networks to the Commission’s Public Consultation on Rare Diseases (February 2008), based on work performed throughout 2007 in this field through Eurordis members and RAPSODY project
- High priority in work plan 2008

### Improving patient access to orphan drugs

- Development of a Position Paper on European collaboration for the Therapeutic Added Value (clinical value) of Orphan Drugs, with scientific common assessment reports. Also served as a specific contribution to the Commission’s consultation on Rare Diseases.
Concept in evolution
High priority in work plan 2008

**Position Paper on Specialised Social Services**
- Development of a Position Paper on Specialised Social Services. This position was developed with patients involved in the Rapsody Project. Also served as a specific contribution to the Commission’s consultation on Rare Diseases.
- Medium priority in work plan 2008

**Calling for political and financial sustainability**
The former idea of a EU High Level Group on Rare Diseases has developed into the proposal for an EU Advisory Committee for Rare Diseases and a possible EU Agency for Rare Diseases
- Eurordis launched a proposal to create an EU Agency on Rare Diseases (or a similar body aimed at ensuring sustainability of activities in the field of rare diseases with a strong focus on research) through its response to the Public Consultation on Rare Diseases launched by the Commission at the end of 2007.
- Eurordis promoted the concept of an EU High level Group on Rare Diseases to be integrated into the policy of the Commission in public health, research and health products with the policy of Member States and patient organisations. This concept has now evolved into the proposal for an EU Advisory Committee for Rare Diseases (a much stronger body replacing the Rare Disease Task Force).

**Information to patients**
- Eurordis comments on the European Commission’s Draft Report on current practice with regard to the provision of information to patients on medicinal products (June 2007)
- Medium priority in work plan 2008
- Work through European Patient Forum and EMEA Patient and Consumer Working Party

**Contributing to the future of pharmaceuticals**
- Contribution to the Commission’s consultation on the future of the EU single market in pharmaceuticals for human use (October 2007)
- Medium priority in 2008

**Advocacy to get the Marketing Authorisation for Drugs having it for a common indication but not for the rare one**
- Advocacy activities towards certain big pharmaceutical companies for specific products and in the framework of the European Federation of Pharmaceutical Industries (EFPIA) Think Tank.
- Medium priority in work plan 2008
Organ transplantation
- Eurordis identified as a key stakeholder in this field. Position paper issued in 2006 had an impact on policy proposal
- Medium priority in work plan 2008

Animal experimentation for Health
- Meetings on this issue and identification of key people within the membership to tackle this issue
- Low priority in work plan 2008
- Work through European Patient Forum

International Relationship development
- In the USA: National Organisation for Rare Disorders (NORD), Genetic Alliance, the Food and Drug Administration (FDA) and the National Institute of Health (NIH)
- International Conference for Orphan Drugs and Rare Diseases (ICORD). Eurordis has contributed to the creation of ICORD, its mission statement and its by laws. Eurordis is a founding member and member of its Board of Directors. The main aim is to organise an international conference every two to four years and to promote rare disease and orphan drugs as a priority in all continents, to encourage international collaboration.

Eurordis is present in European institutions and platforms
European institutions:
- EMEA Committee for Orphan Medicinal Products (COMP)
- EMEA Patients and Consumers Working Party (PCWP)
- COMP Working Group with Interested Parties (WGIP)
- Rare Disease Task Force at DG Health and Consumer Protection
- EU Health Policy Forum at DG Health and Consumer Protection (EHPF)

European platforms:
- European Patients’ Forum (EPF) is the umbrella group of pan-European patient groups active in the field of European public health and health advocacy
- European Platform for Patients’ Organisations, Science, and Industry (EPPOSI) EPPOSI is a European patient-led partnership between patients, industry and academia, founded in 1994, for the exchange of information and discussion of policies in EU human healthcare
- International Alliance of Patients’ Organizations (IAPO)
- European Federation of Pharmaceutical Industries and Associations (EFPIA) EFPIA represents the research-based pharmaceutical industry operating in Europe. Eurordis is part of the EFPIA Think Tank, which is the working group between European patient umbrella groups and industry
- European Forum for Good Clinical Practice (EFGCP)
- Pan-European Blood Safety Alliance (PBSA)
Other contacts

- Many meetings took place in 2007 with Novartis, GSK, Pharmion and cancer patient organisations to build a relationship and a common agenda in the field of rare cancers.
Committee of Orphan Medical Products (COMP) at the EMEA

- 2 full members and 1 observer
- 11 meetings at the EMEA, London
- 125 new applications in 2007
- 98 new designated orphan drugs in 2007
- 15 new market authorisations in 2007

Orphan drugs: more, better, faster

- 4th Eurordis survey on access to orphan drugs in Europe conducted in conjunction with COMP members, EBE-EuropaBio, MEDEV, National Competent Authorities
- Close collaboration with EMEA and FDA to create a common EU/US format for orphan drug applications
- Promotion of a new EU incentive: FP7 clinical research grants for designated orphan drugs

Clinical trials

- The "Eurordis Charter for Collaboration between Sponsors and Patient Organisations for Clinical Trials in Rare Diseases", which was adopted in 2006, has not been applied yet. However, patient representatives have been empowered through the trainings in clinical trial protocols that were organised under the CAPOIRA project.

Paediatric Committee

- Process to identify and select Eurordis’ patient representatives candidates for the future EMEA Paediatric Committee (PDCO) based on volunteer job descriptions, calls for expression of interest, selection by the Board

Objectives:

- Develop rare disease treatments and orphan drugs
- Expand Eurordis’ activities in drug development beyond orphan drugs. Progressively improve coverage of the whole spectrum of patient needs and long term capacity. Take advantage of scientific and medical progress.
- Promote future EU Regulation for paediatric drugs
- Empower rare disease patient organisation representatives in clinical research activities
- Develop patient driven research activities in Europe
Development and submission of a comprehensive application for the Eurordis nominees
Support of Eurordis volunteers to build their capacities and network in paediatric drugs

Eurordis Round Table of Companies (ERTC)
- 8 new members in 2007:
  - **Emerald level**: Chiesi; Farmaceutici S.p.A.; GlaxoSmithKline; Helsinn Healthcare SA; IDM Pharma, Inc.; Janssen-Cilag France and Pfizer
  - **Sapphire level**: Amicus Therapeutics, Inc.; ERYtech Pharma
- 2 upgrades:
  - Aspreva Pharmaceuticals from Sapphire to Emerald
  - Novartis from Emerald to Ruby
- 33 members in total
- 2 successful workshops:
  - 09 July 2007, Barcelona: 61 attendees from 14 countries
    - "Do Rare Disease Patients Have Real Access to Orphan Drugs in Europe?"
    The 4th Eurordis survey on access to orphan drugs was presented during this workshop. As in the previous surveys, this 4th survey showed unequal access to orphan drugs across Europe. As a result of this workshop, Eurordis is putting forward concrete proposals for actions at the EU level that will benefit patients first, as well as all parties.
  - 14 December 2007, Paris: 57 attendees from 10 countries
    - "Proof of Concept and Level of Evidence in Orphan Drug Development"
    For this technical workshop, representatives from the USA and Europe, as well as the UK, France, Germany national regulatory agencies, the industry and patient organisations were invited to present their views on the definition of proof of concept and their experience. One of the conclusions was that it is more appropriate to talk about "proofs of concept" as different levels of evidences are required at different stages of the orphan drug development.

Rare Diseases as a research priority
- Representation of rare disease patients at the DG Research. Workshop on Rare Disease Research priorities on June 14, 2007 in Brussels.
- **European Conference on Rare Disease Research “Building on Success” on September 13, 2007** in Brussels. Participants from a broad range of countries and stakeholders. Eurordis was a member of the Programme Committee and a key note speaker. This conference played an important role in promoting rare diseases on the EU research agenda for the next years.
- **E-Rare** - E-Rare is the Era-Net project that has been established to develop synergies between the eight national research programmes on rare diseases in the European Union, and to set up a common research policy on rare diseases. Eurordis is not a partner, but a member of the External Advisory Board together with EMEA, COMP, EBE, EFPIA. In 2007, E-Rare launched the first joint call for proposals for research on rare diseases.
The EuroBioBank Network: a new step forward

- Since January 2007, EuroBioBank has been officially involved in the new TREAT-NMD Network of Excellence (Translational Research in Europe – Assessment and Treatment of Neuromuscular Diseases). As most members of EuroBioBank are involved in neuromuscular research, this network forms the basis of the neuromuscular biobanking activities within TREAT-NMD. Eurordis, Partner 11, is leader of WP04.1 on “Develop and Manage Supranational Biobanks”.

- As a consequence of its involvement in the TREAT-NMD Network of Excellence, EuroBioBank will be supported by FP6 EU-funding for the next 5 years (2007-2012)

- 16 publications resulting from scientific work performed with samples from the network in 2007

- As EuroBioBank coordinator, Eurordis was actively involved in the preparation of the Biobanking and Biomolecular Resources Research Infrastructure (BBMRI) the new European project aiming at networking all existing and future biological resource centres and infrastructures

Participation in EU research projects

European Clinical Research Infrastructures Network (ECRIN)

- 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.

- Eurordis and ECRIN are collaborating in the CAPOIRA project, of which Eurordis is partner

CliniGene

- European network for the advancement of clinical gene transfer and therapy. Eurordis is involved as a member of the Ethical Review Board.

STEM-HD

- A Specific Targeted Research Project (STREP) funded by the European Commission within its FP6 Programme under the thematic area "Life Sciences, genomics and biotechnology for health", contract number LSHB-CT-2006-037349.STEM-HD. This three year research programme, aims to make major contributions to the understanding of the molecular mechanisms of Huntington’s disease as a necessary step towards a cure.
Board of Directors

- 4 meetings of the Board of Directors and 5 of the Board of Officers to address Eurordis’ strategic issues: Work plans 2007 and 2008; orientations 2007-2009; review of national policies, European Public Affairs, Centres of Expertise and European Reference Networks, Therapeutic Development, Follow-up on the Charters of good practice for on-line communities and for relationship between patients groups and sponsors of rare disease clinical trials, new European services for patients, international orientations, volunteers, budget development and sources of funding.

- 4 new board members were elected at the time of the General Assembly in Paris in May 2007 and 1 member stepped down. The Board comprises 12 members of 8 European countries (Germany, Denmark, Spain, France, Greece, Italy, Romania and Sweden). 10 out of 12 are patients or parents of patients. All of them represent patient organisations.

Staff replacements

- Recruitment of Communications Manager to succeed previous one
- Launch of recruitment of Drug Development Program Manager
- Launch of recruitment to replace Web Communication Manager on sabbatical leave

- Total number of staff at 31 December 2007: 18 persons, 16 full-time equivalent (FTE)

Organisation

- Accounting and monthly financial reporting in a timely manner
- IT and office support upgraded
- Investment in the development of a comprehensive Contact Database to improve quality of interaction and contact management with members, volunteers, partners and stakeholders.
EU project applications to DG SANCO

- Patients’ Consensus on Preferred Policy Scenarios for Rare Diseases (POLKA) project, which aims at involving patients and their representatives in the process of defining strategies and plans for rare diseases that the European Community and the Member States should undertake in a near future. This new project also includes the continuation on the work on centre of expertise and European Reference Networks and the next European Conference on Rare Diseases 2010 Poland. The application was successfully evaluated and accepted although with less funding than requested. Contract signed in 2008.

Eurordis is a partner in 2 project applications:

- The European Project for Rare Diseases National Plans Development (EUROPLAN), which aims at developing recommendations on how to define a strategic plan for rare disease at national level and to promote such plans. Eurordis is the only private partner along 19 Member States. Contract signed in 2008.

- The European Network of Reference for Rare Paediatric Neurological Diseases (nEUroped) which aims to develop a network of communication and information sharing across the field of rare nervous system disorders in children characterised by paroxysmal attacks. Negotiations still pending.

Fundraising and partnerships

- The principles of a renewed contract were agreed, including new provisions (2007-2009), on a 3-year basis in May 2002 between the AFM and Eurordis. This includes the provision of core funding, office space and scientific staff.

- Adoption of a fundraising and partnership development strategy after an Ad Hoc Working Group of Board and staff members worked for six months with a consultant.

- Application submitted to the Medtronic Foundation for the RareTogether project was successful (2008-2010). The project is meant to improve the capacity of rare disease patient organisations to build and operate European disease-specific federations.

- Application for the Eurordis Summer School for patient advocates in drug development and regulatory affairs submitted to the Drug Information Association (DIA) Philanthropy was successful (2008-2009).

Volunteer base

- 273 volunteers in total involved in Eurordis’ activities during 2007
  - 182 regular/long-term volunteers
  - 60 volunteer translators
  - 31 mailing lists moderators
APPENDICES

- Board of Directors and Board of Officers
- Members of Eurordis
- Governance
- Staff Organisation
- Revenue and expenses 2007
- Acknowledgements
None of the directors of Eurordis receive any financial compensation for their duties – although the time they commit to Eurordis amounts to several days per month or even per week in some cases. Directors are reimbursed the expenses they incur upon justification.

The Board of Directors comprises 12 directors, who are all rare disease patient group representatives mandated by their organisations; 5 of them are patients and 5 are parents of people affected by a rare disease; and 2 are professionals. They are from 8 European countries.

- Mrs Abbey Meyers, NORD, National Organisation for Rare Diseases, USA (Honorary President)
- Mr Terkel Andersen*, Danish Haemophilia Society, Denmark (President)
- Mr Pierre Birambeau, AFM, French Muscular Dystrophy Association, France
- Ms Dorica Dan, Romanian Prader Willi Association, Romania
- Ms Susana Díaz Rubiales, APAC, Spanish Association for Growth, Developmental and Lysosomal Disorders, Spain
- Mr Jean Elie*, French Cystic Fibrosis Organisation, France (Treasurer)
- Mr Torben Grønnebæk, Rare Disorders Denmark, Denmark
- Mrs Marianna Lambrou, Tuberous Sclerosis Association of Greece, Greece
- Mr Flavio Minelli*, UNIAMO, Italian Federation for Rare Diseases, Italy (Officer)
- Mr Harald Niemann, GBS Initiative e.V., Germany
- Mrs Christel Nourissier*, Prader Willi, France (General Secretary)
- Mr Anders Olauson, Agrenska, Sweden
- Mrs Rosa Sanchez De Vega*, FEDER, Spanish Rare Disease Federation (Vice President)

(* Members of the Board of Officers)
**Members of Eurordis**

**Eurordis Members 2007**

**Argentina**
Fundacion GEISER - Grupo de Enlace, Investigacion y Soporte Enfermedades Raras

**Armenia**
Doctors and children health care
Neurohereditary Diseases Charity Association

**Austria**
European Haemophilia Consortium
European Network for Research on Alternating Hemiplegia
Pulmonary Hypertension Association Europe (PHA Europe)
Selbshilfegruppe Lungenhochdruck - Austrian PH Patient Group

**Belgium**
Association Belge du Syndrome de Marfan asbl
Association de Patients souffrant d'Hypertension Artérielle Pulmonaire en Belgique - HTAP Belge Asbl
Association de soutien et d'aide aux familles concernées par le syndrome de microdélétion 22q11.2
Association Spina Bifida Belge Francophone
Belgische Organisatie voor Kinderen en Volwassenen met een Stofwisselingsziekte
Euro Ataxia - European Federation of Hereditary Ataxias
European Myeloma Platform
FEWS - Federation of European Williams Syndrome
Groupe d'Entraide Belge du Syndrome Gilles de la Tourette
HAE Belgium

**Bulgaria**
Information Centre for Rare Diseases and Orphan Drugs - Bulgarian Association for Promotion of Education
National Alliance of People with Rare Diseases (NAPRD)

**Burkina Faso**
Fondation Internationale Tierno et Mariam

**Canada**
Canadian Organization for Rare Disorders

**Cyprus**
Thalassaemia International Federation (TIF)

**Czech Republic**
Czech Huntington Society
Czech Society of Haemophilia (Český svaz hemofiliků)

**Denmark**
Danish Apert Syndrome Association /Danmarks Apertforening
Danish Cystic Fibrosis Association
Danmarks Blodforening / Danish Haemophilia Society
Ehlers-Danlos Foreningen i Danmark
Landsforeningen AF ARM-/BENDEFEKTE
Landsforeningen for Blaereektopi Patientforeningen HAE Danmark
Porfyriforeningen Danmark - Porphyria Association Denmark
Rare Disorders Denmark (Sjaeldne Diagnoser)
The Danish Osteogenesis Imperfecta Society
Finland
Finnish Association of People with Mobility Disabilities
Rare Neurological Diseases / Finnish MS Society
The Finnish Association of Societies for Persons with Intellectual Disabilities

France
AIDES
Alliance Maladies Rares
Alliance Sanfilippo
Association Amylose_Info
Association Anémies
Dyserythropoïétiques Congénitales
Association Contre les Maladies Mitochondriales
Association de Lutte contre les Maladies à Prions
Association de soutien à la Recherche pour le traitement Métabolique et Génétique des Homocystinuries
Association des Amis d’Anne-Lorène - Syndrome d’Aicardi
Association des Groupes Amitié Turner
Association des Malades des Vaisseaux du Foie
Association des Malades du Syndrome de McCune-Albright
Association des Malades d’un Syndrome Néphrotique
Association des Malades souffrant d’Angio Oedème par déficit en C1 inhibiteur
Association des Pancréatites Chroniques Héréditaires
Association des Patients de la Maladie de Fabry
Association des Personnes concernées par le Tremblement Essentiel
Association des POIC
Association des Sclérodermiques de France
Association du Locked-in Syndrome
Association du Naevus Géant Congénital
Association du Strümpell-Lorrain
Association Française contre les Myopathies
Association Française de la Dyskératose Congénitale (AFDC)
Association Française de la Maladie de Behcet
Association Française de l’Ataxie de Friedreich
Association Française de l’Ostéogénèse Imparfaite
Association Française de Narcolepsie-Cataplexie et Hypersomnie
Association Française des Dysplasies Ectodermiques
Association Française des Hémophiles
Association Française des Syndromes d’Ehlers-Danlos
Association Française du Gougerot-Sjögren
Association Française du Lupus et autres Maladies Auto-immunes
Association Française du Syndrome d’AICARDI
Association Française du Syndrome de Cornelia de Lange
Association Française du Syndrome de Marfan
Association Française du Syndrome de Rett
Association Française du Syndrome d’Evans
Association Française du Syndrome d’Ondine
Association Française Lesch-Nyhan Action
Association Française pour le Syndrome de Gilles de la Tourette
Association François AUPETIT
Association Francophone contre la Polychondrite Chronique Atrophianté
Association Francophone des Glycogénoses
Association Hereditary Non Polyposis Colon Cancer France
Association Histioyctose France
Association Huntington France
Association internationale de dystrophie Neuro Axonale Infantile
Association Kourir
Association Médicalistes
Association Nationale des
Cardiaques Congénitaux
Association Nationale du Syndrome X Fragile "Le Goëland"
Association Neurofibromatose & Recklinghausen
Association pour la Prévention, le Traitement et l'Etude des Polyposes Familiales
Association pour la Recherche sur l'Atrophie Multisystématisée
Information-Soutien en Europe
Association pour l'Information et la Prévention de la Drépanocytose
Association PSP France
Association Sclérose Tubéreuse de Bourneville
Association SOS Desmoïde
Association Spina Bifida et Handicaps Associés
Association Surrénales
Association Syndrome de Rokitansky - MRKH
Charcot-Marie-Tooth France
Connaître les Syndrômes Cérébelleux
Cutis Laxa Internationale
Duchenne Parent Project
France
Enfants de la Lune Association pour le Xeroderma Pigmentosum
European Federation of Associations of Patients with Haemochromatosis
Fédération des Malades Drépanocytaires et Thalassémiques
Fédération Nationale des Associations Huntington Espoir
France
Lymphangioliomyomatose (FLAM) Generation 22
Genespoir: Association française des Albinismes
GENIRIS
HTAPFrance
Hypophosphatasie Europe
La Chaînette
L'Envol pour les enfants européens
Les Enfants du Jardin
Ligue contre la cardiomyopathie
Ligue Nationale contre le Cancer

L'Oiseau Bleu
Lupus France
Mosaïques - Association des "X Fragile"
Naevus 2000 France-Europe
Prader Willi France
RETINA France
Syndrome de Moebius France
Union Nationale des Associations Parents et Amis personnes Handicapées Mentales
Vaincre la Mucoviscidose
Vaincre les Maladies Lysosomales
Valentin - Association des Porteurs d’Anomalies Chromosomiques

Germany
ACHSE Allianz Chronischer seltener Erkrankungen e.V.
aktion benni & co e.V.
Bundesselbsthilfevereinigung
Multiple Kartilaginäre Exostosen (Osteochondrome) e.V.
Bundesverband Kleinwüchsiges Menschen und ihre Familien e.V.
Cystic Fibrosis Europe
Deutsche Gesellschaft für Osteogenesis Imperfecta (Glasknochen) Betroffene e.V.
Deutsche Sarkoidose Vereinigung gemeinnütziger e.V.
Deutsche Selbsthilfe angeborene Immundefekte eV
Deutsche Uveitis-Arbeitsgemeinschaft eV
Erwachsenen-Histiozytose X e.V.
European Association of Patient Organisations of Sarcoidosis (EPOS)
European Chromosome 11Q Network
Forscher - und Patientengruppe Chorioideremie
Gaucher Gesellschaft Deutschland e.V.
Gesellschaft für Mukopolysaccharidosen e.V.
Guillain Barré Syndrom - Bundesverband GBS Initiative e.V.
HAE Vereinigung e.V.
(Hereditary AngioEdema)  
Hoffnungbaump e.V.  
ICA-Deutschland e.V.  
Förderverein Interstitielle Cystitis  
IEB/DEBRA Deutschland  
Kindernetzwerk für Kranke und  
behinderte Kinder und Jungendliche  
in der Gesellschaft  
Kindness for Kids Foundation  
Kreis für Eltern von Kindern mit  
Speiseröhrenmissbildungen e.V.  
Leben mit Behcet -  
Süddeutschland  
Leben mit Behcet in  
Deutschland  
Mastozytose Internet  
Selbsthilfegruppe  
Myeloma Euronet - European  
Network of Myeloma Patient Groups  
NCL-Gruppe Deutschland e.V.  
OIFE - Osteogeneis Imperfecta  
Federation Europe  
Pro Retina Deutschland e.V.  
pulmonale hypertonie e.V.  
Selbsthilfegruppe Ichthyose e.V.  
Selbsthilfegruppe Ektodermale  
Dysplasie e.V.  
Selbsthilfegruppe für PXE -  
Erkrankte Deutschlands e.V.  
Selbsthilfegruppe für Menschen mit Anorektalfehlbildungen  
Sklerodermie Selbsthilfe e.V.  

**Greece**  
Association of people with  
genetic abnormalities  
Greek Alliance for Rare Disease  
Tuberous Sclerosis Association of Greece  

**Hungary**  
Federation of NGOs of people  
with Chronic Illnesses  
Rare Diseases Hungary -  
Hungarian Federation of People with  
Rare and Congenital Diseases  

**Iceland**  
Einstökbörn - Support Group  
for Children with Rare Disorders  
Hypoparathyroidism Europe  
(HPTH Europe)  
The Icelandic Childhood Cancer  
Parent Organization  

**Ireland**  
Alpha One Foundation  
Fighting Blindness  
Genetic and Inherited Disorders  
Organisation (GIDO)  
Huntington's Disease  
Association of Ireland  
Irish Ants Co. Ltd.  
(Syringomyelia Self Help Group)  
Irish Motor Neurone Disease  
Association  
Muscular Dystrophy Ireland  
Neurofibromatosis Association  
of Ireland  

**Italy**  
Associazione Antonio Pinzino  
Onlus  
Associazione Del Bambino  
Emopatico  
Associazione Famiglie di  
Soggetti con Deficit dell'Ormone della  
Crescita e sindrome di Turner  
Associazione Internazionale  
Ring 14  
Associazione Italiana Cistite  
Interstiziale  
Associazione Italiana  
Glicogenosi  
Associazione Italiana  
Kartagener / PCD  
Associazione Italiana Miastenia  
Onlus  
Associazione Italiana  
Mucopolisaccaridosi e Malattie Affini  
Associazione Italiana Sclerosi  
Laterale Amiotrofica (sez. Lombardia)  
Associazione Italiana sindrome  
e malattia di Behçet (SIMBA)  
Associazione Italiana Studio  
Malattie Metaboliche Ereditarie Onlus  
Associazione Malati di Porfiria  
Associazione Nazionale Italiana  
Malati Sindrome di Sjogren  
Associazione per la Ricerca  
sull'Epidermolisi Bollosa Distrofica  
(DEBRA Italia)  
Associazione per l'Informazione  
e Lo Studio della Acondroplasia  
Associazione Sclerosi Tuberosa  
Associazione Studio Malattie  
Metaboliche Ereditarie ONLUS  
Associazione Veneta per la  
Lotta alla Talassemia  
Federazione delle Associazioni  
per l’Aiuto ai Soggetti con Sindrome di
Prader Willi
Parent Project ONLUS
UNIAMO - Federazione Italiana Rare
Malattie Rare
Unione Italiana Ittiosi
Visus - Associazione tra Affetti
da Retinite Pigmentosa

Luxembourg
Association Luxembourgeoise
d'aide pour les personnes Atteintes de maladies Neuro-Musculaires et de
Een Häærz fir kriibskrank
Kanner asbl

Malta
EAMDA European Alliance of
neuroMuscular Disorders
Associations

Netherlands
Autosomaal Dominant
Cerebellaire Ataxie-Vereniging
Nederland
Fabry International Network
International Painful Bladder
Foundation
Nederlandse Vereniging voor
Addison en Cushing Patiënten
(NVACP)
Stichting Langerhans Cel
Histiocytose
Stichting voor
AfweerStoornissen
Vereniging Samenwerkende
Ouder en Patiëntenorganisaties
(VSOP)
Vereniging voor Kinderen met
Stofwisselingsziekten
Vereniging voor mensen met
het Van Lohuizen syndroom

New Zealand
New Zealand Organisation for
Rare Disorders

Norway
FRAMBU - National Center of Rare Disorders
Nordic HPTH Organisation
Norsk Forening for Ehlers-Danlos syndrom

Poland
MATIO-Fundacji Pomocy
Rodzinom i Chorym na
Mukowiscydozę

Portugal
Associação Nacional de Fibrose Quística
Associação Portuguesa de Doentes Neuromusculares
Liguia Nacional para o Estudo e Apoio da Deficiência Mental
Núcleo de Epidermolise

Romania
Asociatia Williams Syndrome
Romanian Prader Willi Association

Slovenia
Foundation of Child Neurology

Spain
Alianza Española de Familias de Von Hippel Lindau
Asociación Catalana de las Neurofibromatosis
Asociació d'Afectats de Siringomièlia
Asociación Andaluza contra la Fibrosis Quística
Asociación Andaluza del Síndrome de Gilles de la Tourette
Asociación Balear de Afectados por la Trigonitis y la Cistitis Intersticial
Asociación de Deficiencias de Crecimiento y Desarrollo
Asociación de Escleroderemia Castellon
Asociación de Hemofilia de la Comunidad de Madrid
Asociación de Huesos de Cristal de España
Asociación de las Mucopolisacaridosis y síndromes relacionados
Asociación de Nevus Gigante Congéntito
Asociación de Pacientes de la Enfermedad de Huntington
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
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<td>Asociación Sindrome Angelman European Network for Rare and Congenital Anaemias</td>
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<td>The Swedish Association of Rare Disorders (Riksförbundet Sälsynta diagnoser)</td>
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<td>The Swedish Cooperative Body of Organizations of Disabled People</td>
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<td>Romande et Italien contre les Myopathies</td>
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<td>Taiwan</td>
<td>Taiwan Foundation for Rare Disorders</td>
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<td>Kharkiv’s charitable Foundation - &quot;Children with spinal muscular atrophy&quot;</td>
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<td>Children Living with Inherited Metabolic Diseases</td>
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<td>International Brain Tumour Alliance</td>
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<td>IPOPI - International Patient Organization for Primary Immunodeficiencies</td>
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<td>Niemann-Pick Disease Group</td>
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<td>Stiff Man Syndrome support group and charity</td>
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<td>Sturge-Weber Foundation UK</td>
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<td>The Jennifer Trust for Spinal Muscular Atrophy</td>
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<td>The Society for Mucopolysaccharide Diseases</td>
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<td>Tuberous Sclerosis Association</td>
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<td>Tuberous Sclerosis Europe</td>
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<td>Unique - The Rare Chromosome Disorder Support Group</td>
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WellChild

USA
NORD National Organization for Rare Disorders
Governance

Over 182 regular volunteers from many countries and rare diseases, various patient group sizes and competences, contribute to Eurordis’ activities.
ACRONYMS

- **CHMP PCWP**: EMEA Committee for Medicinal Products for Human Use – Patients’ and Consumers’ Working Party
- **COMP**: EMEA Committee for Orphan Medicinal Products (designation of an orphan product)
- **COMP WGIP**: EMEA COMP Working Group of Interested Parties
- **Clinigene**: EU network for the advancement of clinical gene transfer and therapy
- **Comité National de Labellisation**: French National Committee on Accreditation of National Centres of References
- **DIA EuroMeeting**: European Annual Meeting of the Drug Information Association
- **EBE**: Emerging Biopharmaceuticals Enterprises
- **ECRIN**: European Clinical Research Infrastructures Network
- **EFPIA**: European Federation of Pharmaceutical Industries
- **EMEA**: European Medicines Agency
- **EPF**: European Patients' Forum
- **EPPOSI**: European Platform for Patients’ organisations, Science and Industry
- **E-Rare**: Develop Synergies for 8 national research programmes on rare diseases in Europe
- **EuroBioBank**: European Network of DNA, Cell and Tissue Banks for Rare Disease Research
- **Haute Autorité de Santé**: French governmental body – High Health Authority for hospitals accreditation and health evaluation
- **IAPO**: International Alliance of Patients’ Organizations
- **ICORD**: International Conference On Rare Diseases & Orphan Drugs
- **INSERM**: French national institute for health and medical research
- **Maladies Rares Info Services**: French Helpline service for rare diseases
- **PBSA**: Pan-European Blood Safety Alliance
- **Plan National Maladies Rares**: French National Plan for Rare Diseases
- **Treat-NMD**: Treat Neuro Muscular Diseases research project
Staff Organisation

The team comprises 18 people, 16 FTE as of December 2007. The team is made of paid staff, volunteers, trainees, consultants and free-lance staff. Eurordis’ European Public Affairs Officer and European, Public Affairs Advisor are based in the Brussels office.

ORGANISATIONAL CHART
2007

Yann Le Cam
Chief Executive Officer
1/1
BoD liaison:

Ariane Weinman
1/1
Personal Assistant

Flaminia Macchia
3/5
Director

Research & Therapeutic Development

Valentina Bottarelli
3/5
Advisor

Fabrizia Bignami
1/1
Director Therapeutic Development and Research

François Faurisson
1/1
Clinical Research Advisor

To be recruited
1/1
Drug Dev Prog Manager* (Started on 7th January

Anne Mary Bodin
1/1
Project Assistant EuroBioBank & ERTC

Simone Keita
2/5
Volunteer

Research & Therapeutic Development

Françisco Houyéz
1/1
Director Health Policy

Health Policy & Networking

Anja Helm
4/5
Manager of Relations with Patient Organisations

Shane Lynam
1/1
Project Coordinator

Communications & Development

Kasia Peala
1/1
Project Assistant

Julia Fitzgerald
4/5
Manager of Web

Paloma Tejada
1/1
Communications Manager

Nathacha Appanah
4/5
Communications Assistant

To be recruited
1/1
Director Communications & Development

Communications & Development

Patrice Régnier
1/1
Director Finance & Support Services

Eugénie Cagnac
1/1
Assistant Admin & Accounting

Annie Rahajantzafy
1/1
Administrative Secretary

Finance & Support Services

Staff Organisation

The team comprises 18 people, 16 FTE as of December 2007. The team is made of paid staff, volunteers, trainees, consultants and free-lance staff. Eurordis’ European Public Affairs Officer and European, Public Affairs Advisor are based in the Brussels office.
Revenue and expenses 2007
(in thousand of euros, provisions excluded)

Revenue 2007 = 2,626

Expenses 2007 = 2,506
Acknowledgements

Eurordis would like to thank the following, organisations and companies for their highly valued support in 2007.

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**AFM - Téléthon**

*Eurordis is grateful to the AFM – Association Française contre les Myopathies (French Muscular Dystrophy Association) for the annual core activities grant, for the secondment of the Therapeutic Development Director of Eurordis and the office space they make available to the organisation free of charge.*

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**EUROPEAN COMMISSION**

*Eurordis is grateful to the European Commission DG Research and DG SANCO for funding 3 Eurordis projects*

- Rare Disease Patient Solidarity Project (RAPSODY)
- Capacity Building for Patient Organisations ain Research Activities (CAPOIRA)
- Treat NMD and Eurobiobank

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**INSERM**

*Eurordis is grateful to the INSERM - Institut National de la Santé et de la Recherche Médicale (French National Institute for Health and Medical Research) for the secondment of the Clinical Research Advisor of Eurordis and for the partnership in training for patient groups.*
RARE DISEASE PATIENT SOLIDARITY PROJECT (RAPSODY)

Eurordis is grateful to the European Commission DG Health and Consumer Protection (DG SANCO), the Portuguese Ministry of Health, the Baxter International Foundation and Sigma Tau Pharmaceuticals for their essential contribution to the RAPSODY project. As well as Novartis and Swedish Orphan for their financial contribution to ECRD 2007 Lisbon for interpretation and local transportation of participants with disabilities.

EURORDISCARE PROGRAMME

Eurordis is grateful to Actelion and UGIM for their essential contribution to the EurordisCare3, which was part of the Rapsody project 2006-2008 for the survey on patients’ experience and expectations on healthcare offer for rare diseases.

THE E-NEWSLETTER

Eurordis is grateful to The Medtronic Foundation for their financial support for the Eurordis E-Newsletter.

MAILING LISTS

Eurordis is grateful to the LEEM – Les Entreprises du Médicament (French Drug Manufacturers Association) for its financial support for the Mailing list project.
PARIS MEMBERSHIP MEETING
Eurordis is grateful to the organisations listed below for their financial support for the Paris Membership Meeting and the European Workshop “Gaining Access to Rare Disease Research Resources”:

With the support of:

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

Emerald Members
Sapphire members

ALEXION Europe
Amicus Therapeutics
Biomarin
BioMarin Europe Ltd.

ERYtech Pharma
The Therapeutic Red Cell Company

MERCK Serono

Neurochem

Jerini
Mark Krueger & Associates, Inc.

Orfagen
Gloucester Pharmaceuticals

ORPHAN EUROPE

PTC Therapeutics

Orphan International

Celgene
Boehringer Ingelheim

GENETHON
NexGenix

Inspired solutions inspire hope