EURORDIS INTERNATIONAL INITIATIVES

200 MILLION FACES,
ONE VOICE FOR RARE DISEASES
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Tens of millions of people around the world are living with a rare disease. Although diseases vary tremendously, people living with a rare disease face many common challenges: lack of information, lack of expertise, lack of treatments, lack of access to treatment and care. Most people living with a rare disease are still health orphans, living outside the scope of public health policies and with limited hopes for a treatment or care.

Addressing rare diseases at an international level is vital to send a message of hope to the millions of patients and families around the world that can only break their isolation through a vast, united international network of solidarity. Many challenges facing the rare disease community at large must be tackled at the international level in addition to national and local ones. We created EURORDIS, the European Organisation for Rare Diseases, in 1997 to provide a united voice for people living with a rare disease in Europe.

Because rare diseases know no borders and because international collaboration for rare diseases is so deeply needed, many of EURORDIS’ actions have grown to have an international reach. EURORDIS strives to empower all people living with a rare disease to reduce the impact of rare diseases through:

- Connecting people living with a rare disease across borders, languages and specific diseases
- Advocating for better rare disease policies – ensuring the patient voice is heard from the initiation to the implementation of public health policies
- Informing and educating patient advocates to make them part of the entire process of developing and accessing treatments for rare diseases

EURORDIS, as a transnational, pan-European, multilingual organisation, feels it must transcend borders and take on international initiatives to bring people together to speak with one united voice. To this end, we are raising €15 million from 2015 -2020 for the initiatives described for you here.

We invite you to support this important work to increase hope for people who are living with a rare disease around the world.

With your help, we can ensure that people living with a rare disease are no longer health orphans in Europe or globally.

Yann Le Cam, Chief Executive Officer, EURORDIS
EURORDIS’ International reach

EURORDIS is a non-governmental, patient-led alliance of rare disease organisations representing nearly 700 patient organisations in over 63 countries throughout Europe and around the world. With your help, EURORDIS can expand its network to support millions more who are living with a rare disease.
The Challenge of Rare Diseases

Rare diseases touch people at every age and within any family. Over 6,000 different rare diseases have been identified to date, affecting more than 60 million people in Europe and the USA alone. Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care often inadequate and research limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.

What is a rare disease?

The definition of a rare disease can vary. For instance, the European Union considers a disease as rare when it affects fewer than 1 in 2,000 citizens. In the USA, a disease is defined as rare if it affects fewer than 200,000 Americans. One rare disease may affect only a handful of patients and another hundreds of thousands of people. More than 50% of rare diseases affect children. About 80% of rare diseases have identified genetic origins.

- Treatments have been developed for less than one-third of rare diseases.
- Access to treatment and care varies widely from country to country and community to community.
- EURORDIS is striving with international partners to improve conditions for people living with a rare disease, so that by 2020:
  - Almost all rare diseases are identified and described.
  - Diagnostic tests exist for over 4,000 rare diseases.
  - At least 200 new rare disease therapies are approved – bringing the total number of rare disease therapies to 500 or more.
  - Rare diseases are recognised as a public health priority worldwide.
  - Registration of patients and systematic collection of data is the rule to generate new knowledge about the diseases, identify practices of care and compare health outcomes.
  - Anyone with internet access living with a rare disease can find a supportive community of people facing many of the same challenges, regardless of the language they speak.
EURORDIS’ leadership capacity to drive this change: KEY ACHIEVEMENTS

When EURORDIS started in 1997, the concept of rare diseases was ignored in Europe. Only fragmented approaches were explored, and then only for a few of the thousands of rare diseases that exist. We created EURORDIS to bring diverse people with rare diseases together to speak with one strong voice across all rare diseases and all European countries. We still have a long way to go before all people with a rare disease have equal access to treatment and care, but we now have the momentum to build upon.

Rare diseases are included as a public health priority for Europe. The improved legal framework we continue to fight for has assisted more than 100 treatments for rare diseases to receive marketing authorisation in the EU; national plans for rare diseases are being developed across Europe and beyond; and most importantly, people living with a rare disease have become a critical mass of citizens to no longer be ignored.

EURORDIS has a robust track record operating in 48 European countries and in 7 languages. It is widely recognised in Europe for helping authorities understand why rare diseases are a public health priority, why rare diseases require a specific legal framework and why patients themselves must have a voice in creating and implementing policies and research. As such, EURORDIS is a main partner working with the European Commission, the European Parliament, the European Council, the European Medicines Agency (EMA), EUnetHTA and other stakeholders, including Orphanet, the European Federation of Pharmaceutical Industries and Associations (EFPIA), the Drug Information Agency (DIA), European biopharmaceutical enterprises, among others.

In particular, EURORDIS’ contributions to the following demonstrate how patient organisations can shape policy:

- The designation of over 1,100 orphan medicinal products
- The placement of the first patient as a voting member of an EMA committee
- The subsequent placement of 9 patient representatives on European Union committees making decisions on rare diseases
- The adoption of the EU Regulation on Orphan Medicinal Products in 1999
- The adoption of the EU Regulation on Paediatric Drugs in 2006
- The adoption of the EU Regulation on Advanced Therapy Medicinal Products in 2007
- The adoption of the EU Commission Communication Rare Diseases: Europe’s Challenges in 2008
- The adoption of the EU Council Recommendation on a European action in the field of rare diseases in 2009
- The adoption of the EU Directive on Patients’ Right to Cross-Border Healthcare in 2011
- The promotion and maintenance of rare diseases as:
  - EU public health policy priority
  - EU research framework programme priority
- The advancement of national plans and strategies on rare diseases in all 28 EU Member States and other European countries
EURORDIS INTERNATIONAL INITIATIVES

EURORDIS is striving to empower all people living with a rare disease by:

**Connecting people**

Loneliness and lack of information are hardships known to many people living with a rare disease. EURORDIS works to strengthen rare disease communities across borders, across languages and across diseases.

Initiative:
- RareConnect Online Rare Disease Communities

**Advocating and building awareness**

EURORDIS works to ensure that patients have a strong voice not only in the development of health policies, but also in their implementation.

Initiatives:
- Patient Voices
- Rare Diseases International (RDI)
- Rare Disease Day

**Informing and educating**

Face-to-face training programmes, online forums and information from trustworthy resources in multiple languages are all needed to connect and inform patients and families across borders.

Initiatives:
- EURORDIS training programmes, including the EURORDIS Summer School
- EURORDIS eNews, Member News, social media channels & eurordis.org

I contribute to EURORDIS because I’ve seen the amazing impact of empowered patient advocates. I’m proud to support this work at the international level.

Claudia Hirawat, Chair of the EURORDIS International Circle of Ambassadors
Two of the greatest challenges for people living with a rare disease are social isolation and lack of access to quality information about their disease. RareConnect Online Rare Disease Communities transcend borders, language barriers and diseases to break the isolation that often comes with a rare disease.

Thousands of rare disease patients and families from around the globe are building communities, sharing experiences and accessing quality information via Rareconnect.org. Visited regularly by more than 100,000 visitors and endorsed by over 600 patient organisations, RareConnect is home to more than 75 international communities. Each rare disease community is created with patient organisations that endorse and promote the community, and that provide volunteer moderators who receive ongoing support and training from EURORDIS staff. Moderators’ skills and commitment ensure the safety and relevance of their community.

This structure fosters a secure environment where patients can connect, share their experiences and learn about their disease.

What sets RareConnect apart from other online patients communities?

- **RareConnect is specific to rare diseases.** RareConnect enables rare disease patients to find and exchange information relevant to them more quickly and easily.
- **RareConnect enhances the role of patient organisations.** Members of patient organisations design, develop and moderate their own communities.
- **RareConnect is not-for-profit.** Patients’ privacy is guarded and data is secured by EURORDIS.
- **RareConnect has a truly international reach.** Rare disease patient organisations in the United States, Europe and around the world create online communities via RareConnect.
- **RareConnect transcends language barriers.** The site is published in 5 languages and offers custom translation on demand.
- **RareConnect provides quality content.** Each community includes information from vetted sources, allowing community members to find useful, credible information quickly.

"RareConnect is actually how I found my diagnosis! I posted my story on the CAPS community back in 2012."

*Member of the CAPS community*
With your help, in the next 5 years, RareConnect will:

- Host 200 online rare disease patient communities.
- Add 50 topics groups in addition to disease-specific communities to create forums for discussion on issues that transversally affect all rare disease patients.
- Add Portuguese and Russian to RareConnect languages (currently English, French, German, Italian and Spanish).
- Publish a mobile application version of RareConnect.org.
- Welcome 2.6 million unique visitors annually.
- Serve 100,000 active community members, an increasing number from developing nations.
- Recruit and sustain 500 volunteer community moderators.
- Add a surveys function to enable communities to poll their members, in conjunction with Patient Voices.

**RARECONNECT STATISTICS**

**IMPACT**

Your support for RareConnect will:

- Make it possible for more than 100,000 people living with a rare disease to find a community to overcome the isolation brought on by their disease, and to access valid information they need.
- Enable rare disease patient organisations to easily poll members worldwide to better serve the people they represent.
People living with a rare disease are experts in their disease. Nobody else has closer first-hand knowledge of the products and policies developed for rare diseases, or the impact they make on people’s lives. This is why it is vital to include patients’ perspectives when developing and implementing policies on rare diseases.

Patient Voices will gather patient experiences and expectations with validated methods in qualitative and quantitative data collection. At the core of the programme is the development of a database of patients and families.

With the database in place and with its ongoing maintenance and development, EURORDIS will be able to conduct surveys in both a scientific and flexible manner. The programme will be managed by EURORDIS staff specialising in survey methodology applied to political science and sociology, and supported by an advisory committee and ad hoc topic experts from the academic, corporate and public sectors.

EURORDIS members, RareConnect community moderators, researchers and partners will be involved throughout the process, from generating the research question to disseminating the results.

Survey results will be disseminated to EURORDIS Members and via a dedicated section of eurordis.org, in addition to scientific papers published in collaboration with academic partners.

IMpact

Your support for Patient Voices will help:

- Identify common priority issues for people living with a rare disease.
- Produce evidence on topics relevant to current legislation and policy.
- Promote and improve research on patients’ perspectives.
- Ensure that legislation and policy topics are relevant to people living with a rare disease.

The programme’s success will ultimately be seen in the number and quality of health, research and social policies truly reflecting the experiences and expectations of people living with a rare disease.

Engaging the patient voice best informs researchers, clinicians, patients and governments.

Durhane Wong-Rieger, President, Canadian Organization for Rare Disorders
Today, most international rare disease initiatives are ad hoc and linked to one particular disease. People living with a rare disease need to join together through a structured approach to advocacy and exchange to create a global rare disease community. RDI was launched in the spring of 2015 in partnership with rare disease alliances of the US, Canada, Japan, China, India and beyond. RDI is first reaching out to national alliances that federate patient organisations within their countries, international federations of patient groups representing the same disease or the same group of diseases and pan-regional networks for rare diseases. The RDI membership base is developing to include more than 100 patient organisations covering more than 100 countries. RDI members will:

1. Learn from each other through fellowships and exchange among leaders of rare disease patient organisations worldwide.
2. Adopt the joint declaration Rare Diseases: an International Public Health Challenge to advocate for rare diseases as an international public health priority.
3. Develop productive relations with key institutions, including the United Nations through the World Health Organization (WHO) and the Economic and Social Council (ECOSOC)

IMPACT

Your support for RDI will:

1. Unite, expand and reinforce the movement of people living with a rare disease to speak with one strong, international voice.
2. Establish rare diseases as a public health priority in more countries and regions around the world, as well as at the global level.
3. Put rare diseases on the agenda of WHO and other international organisations.
4. Strengthen rare disease patient groups’ capacity to act at local, national, regional and global levels.
Organised yearly around a single theme, Rare Disease Day calls attention to issues common to all people living with a rare disease. Since its beginning in 2008, political momentum from Rare Disease Day has helped advance policies on rare diseases. This can be seen, for example, in the advancement of national plans and policies for rare diseases in a number of countries.

In 2015, Rare Disease Day touched every continent, with rare disease patient organisations holding more than 650 events. The 2015 Rare Disease Day video had over 1.5 million views and was shared across 27 languages. The campaign has progressively become a world phenomenon, with the USA joining in 2009, and participation from a record-breaking 87 countries around the world in 2015.

Rare Disease Day is an integral part of the international rare disease movement and boosts advocacy success worldwide. In the next 5 years, EURORDIS will work with national partner patient organisations to expand the reach of Rare Disease Day, with the following aims:

1. Rare Disease Day events taking place in 160 countries, with an increasing number in developing nations.
2. 100 million people worldwide participating in a Rare Disease Day activity.
3. More than 90 per cent of EURORDIS member organisations reporting that Rare Disease Day helped increase their funding and/or make a concrete advancement in their advocacy agenda.

**IMPACT**

Your support will expand Rare Disease Day, resulting in:

- Increased awareness of rare diseases worldwide.
- Greater solidarity of the international rare disease community, resulting in stronger rare disease patient organisations worldwide.

The last day of February is Rare Disease Day – when hundreds of thousands of individuals and organisations around the world join together to promote awareness of rare diseases and the millions of people affected by them.
Capacity building for patient advocates is vital to ensure patients can play a role in public decision-making bodies, to ensure that patients’ perspectives are fully taken into account by treatment developers and care providers, and to build awareness of real conditions for people living with a rare disease. This is why EURORDIS created the EURORDIS Summer School in 2008. Since then, more than 250 people from over 35 countries have become empowered patient advocates through this 5-day intensive training. Patient advocates and researchers increase their knowledge or learn for the first time many aspects of drug development and regulations at the European level. Among the expert faculty are volunteers from the European Medicines Agency (EMA). Summer School alumni go on to ensure that patients have a strong voice at regional, national and international levels. With your help, EURORDIS can expand its training programmes to include a youth school and topics beyond drug development, build support for alumni and increase participation by researchers. In the next 5 years, EURORDIS is striving to expand its education programmes for people living with a rare disease by:

1. Expanding the training curriculum beyond drug development to include social care.
2. Increasing the reach of the Summer School beyond Europe.
3. Adding a youth programme.
4. Enhancing support of Summer School alumni via ongoing training and networking.
5. Creating an international fellowship programme enabling leaders of rare disease patient organisations to spend a period of weeks with similar organisations in other countries.

**IMPACT**

Your support for EURORDIS training programmes will:
Make it possible for over 250 young rare disease community members and leaders of rare disease patient organisations from around the world to build skills, exchange ideas and form networks that will promote empowerment of people living with a rare disease.

Getting together with other patient representatives - many of them affected themselves with a rare disease - made a great difference in my life. Learning to live with many restrictions and knowing that one is not alone in this effort is one lesson. During Summer School, I found out that it is important to continue with life, to set goals and achieve them, to have fun and dream, even if one has only one minute of life to live.

*EURORDIS Summer School Alumnus*
Communicating across languages is essential to build and sustain a global rare disease movement. Published in 7 languages (English, French, German, Italian, Portuguese, Russian and Spanish), the EURORDIS website, eNews and Member News are a primary source of information for patient advocates, public officials and health industry professionals throughout Europe and internationally.

Each month, more than 7,500 people from patient and public organisations and the health sector receive updates on news from the rare disease community via the EURORDIS eNews. Articles and resources promoted via the eNews are made available on eurordis.org, an online resource accessed by more than 290,000 unique visitors from more than 200 countries each year. Patient advocates worldwide can also make the most of information and documents on the website as examples of how they can take action in their own community. For example, EURORDIS’ toolkit for conferences on national plans for rare diseases is downloaded by visitors from around the world. The most popular section of the website features stories and images of people living with a rare disease worldwide. The website also includes a section for EURORDIS volunteers, promotes relevant events and includes EURORDIS TV, a platform that brings together rare disease videos.

In the next 5 years, EURORDIS is working to increase its international reach via eurordis.org and the eNews by reaching:

1. 10,000 eNews subscribers worldwide.
2. More than 350,000 annual unique visitors to eurordis.org from more than 250 countries.

**IMPACT**

Your support for EURORDIS’ multilingual communications will help:

/math/ensure that people living with a rare disease throughout Europe and the world have access to proven resources to assist them in strengthening their own communities.

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"One day, we will all be ‘rare’.

*Karin and Sean Hepburn Ferrer, EURORDIS Ambassadors*
Supporting EURORDIS’ International Initiatives

EURORDIS is raising 15 million € from 2015 – 2020 to make possible its International Initiatives for people living with a rare disease.

We need to sensitize the general public to the fact that a rare disease is not a marginal phenomenon.

Grâce à notre engagement auprès des patients et de leurs familles, grâce à notre générosité, faisons triompher la vie.

WE NEED YOU! Join us for this solidarity campaign! Be part of this extraordinary challenge!

Tous ensemble, dessinons la grande famille des « Ambassadeurs sans Frontières » d’Eurordis.

Reem Boustany, EURORDIS Ambassador

**A TAILOR-MADE OPPORTUNITY**

We would be honoured to recognise your contribution in a way that best suits you and that maximizes the impact of your gift.

Please contact Jill Bonjean, Resource Development Director for information on how to make your tax-deductible contribution, and how your donation may be recognised.

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EURORDIS thanks participants of the EURORDIS photo contest and programmes, patient advocates and EURORDIS ambassadors whose photos and quotations appear in this document -- people from around the globe who know and care about people living with a rare disease.