The Power of Rare Disease Patient Advocacy

Ariane Weinman, EURORDIS

2 December, 2017
ESMO – RCE – ESO Training Course for Rare Cancer Patients Advocates
Milan, Italy

EURORDIS.ORG
Patients’ Empowerment

Enables patients, families, and patient advocacy groups to:

- Be more and better informed
- Gain trust
- Gain strength in their capacity to act, make choices and transform choices into actions
- Gain more power to take control of their life, their rights, and their social, economic and political conditions
The Concept of Rarity
Rare Diseases and Rare Cancers’ challenges

progressive, degenerative, disabling and life-threatening:

- Patients and experts are **few**, geographically **scattered** and often **isolated**
- Patients are **undiagnosed**, **misdiagnosed** or wait years for a diagnosis
- Reliable **information** is scarce
- Resources are **limited**
- Lack of **treatments and challenges** to access adequate care
- Fragmented research, **data** and information
- High social impact and **marginalisation** within society and within healthcare systems designed for common diseases
- **Heavy psycho-social burden**: societal support is essential to patients and families to enable them to cope, be resilient, care for others

Both children and adults are affected
RARITY CALLS FOR ACTION BEYOND NATIONAL BORDERS

- No one country can solve alone the problems faced by people living with rare diseases
- Need to bring together a critical mass of patients and medical experts, researchers and public health authorities
- To find solutions to provide timely access to diagnosis and adequate treatments, to develop research at European and international levels and to help improve the quality of life of those suffering from rare conditions

Rare diseases: based on prevalence (<50/100,000)
Over 6000

Rare cancers: based on incidence (<6/100,000/year)
About 200, 22% of all cancer cases
ADVOCATING FOR PATIENTS

We advocate to ensure that healthcare and social policies and services, research, and the development of medicines take into account the real needs of people living with a rare disease and their families. Our advocacy is fuelled by real-life experiences of the rare disease community.

EMPOWERING PATIENTS

By uniting the rare disease community and building the capacities of patients, we empower them to become advocates equipped with the knowledge and skills needed to fight for better lives.

ENGAGING PATIENTS

We make possible the engagement of patients in decision-making processes so that the patient voice is active, amplified and meaningful in research and medicines development, as well as in both healthcare and social policies and services.

PATIENTS FIRST
Overcoming fragmentation

Creating a critical mass

Learning from each other

Working together
EURORDIS Community Building

• 761 member organisations in 68 countries

• **Council of 33 National Alliances of Rare Disease Patients**
  → Common Goals & Mutual Commitments, National Plans / Strategies for Rare Diseases & Rare Disease Day

• **Council of 61 European Federations and Networks**
  → Cross border health care, clinical trials for rare diseases, European Reference Networks, registries, access to medicines

• **Membership Meetings**

• **European Conference on Rare Diseases & Orphan Products (ECRD)**
Ensuring Good Governance Processes

MEMBERS
- GENERAL ASSEMBLY
  - Financial Audit Deloitte
- BOARD OF DIRECTORS
  - European Public Affairs Committee
  - EURORDIS Panel of Experts
- BOARD OF OFFICERS
- CHIEF EXECUTIVE OFFICER
  - EURORDIS International Circle of Ambassadors

STAFF

EURORDIS Action Groups & Task Forces
- Therapeutic Action Group (EMA)
- Policy Action Group (Commission Expert Group on RDs)
- Policy Action Group – Rare Cancers (Commission Expert Group on Cancer)
- DITA TF (Drug, Information, Transparency & Access)
- ECRD 2018 Vienna

24 European Patient Advocacy Groups (ePAGs)

EURORDIS STANDING COMMITTEES & COUNCILS
- Council of Rare Diseases International (specific by-laws for autonomous governance)
- Council of National Alliances on Rare Diseases
- Council of European Federations on Rare Diseases
- European Network of RD Help Lines (ENRDHLs)

EURORDIS PROGRAMS & PROJECTS COMMITTEES & WORKING GROUPS
- Health Policy:
  - Social Policy Advisory Group
  - EUROPLAN Advisors
  - RareConnect Steering Committee
  - RareConnect Advisory Committee
  - Rare Barometer Advisory Committee
  - Rare Barometer Topic Experts Committee
- Communication:
  - Editorial Committee
  - Rare Disease Day Steering Committee
  - Black Pearl Evening Committee
- Research & Therapies:
  - RD-Connect Joint Patient Advisory Council
  - Summer School Faculty
- Cross-cutting
  - Operating Grant Steering Committee
  - EURORDIS Membership Meeting 2017 Budapest
Rare Disease Patient Empowerment

Information, Networking, Raising Awareness
Information & Networking

- Website in 7 languages: English, French, German, Italian, Portuguese, Spanish, Russian
- 2x month eNews in 7 languages
- Monthly Member News
- Facebook
- Twitter
- Rare Connect – Patient Online Communities
- Online Surveys: Rare Barometer
- Fact Sheets, Position Papers, Publications
- Annual Awareness Campaign: Rare Disease Day
RareConnect

- Launched in 2010
- Free-access online rare disease patient communities
- 90+ communities in partnership with 661 patient groups
- Supported by 2 full-time community managers and 265 volunteer moderators
- Translators offer free translations between 6 languages (English, French, German, Italian, Spanish, Portuguese, Russian)

- [www.rareconnect.org](http://www.rareconnect.org)

- Questions on starting a community? Contact: [team@rareconnect.org](mailto:team@rareconnect.org)
Rare Barometer: Surveys

High-quality evidence based survey programme capturing the patient voice and experience

- Facilitate and streamline the inclusion of patient perspectives
- Provide a high-quality evidence base
- Support advocacy and policy making activities at EURORDIS
- Promote and improve further research on patient perspectives

www.eurordis.org/voices
Rare Disease Day 2017: 94 countries
Rare Disease Patient Empowerment

Training,
Capacity Building
EURORDIS Summer School (ExPRESS)

- 2008: Launch year, open to patient representatives
- 2015: Inclusion of academic researchers → Expert Patient and Researcher Eurordis Summer School (ExPRESS)
- Over 400 patient representatives and researchers have been trained, coming from 40+ different countries and representing 75+ diseases
- 5-day annual training on:
  - Clinical Trials & Medicines Development
  - EU Regulatory Processes & EMA
  - HTA, Reimbursement, Patient Access
  - Translational & International Research
- Alumni involved in regulatory processes at the EMA and/or in collaboration with sponsors and/or as EURORDIS’ Volunteers
EUPATI, ESO and other training courses

- **EUPATI**: 'European Patients Academy on Therapeutic Innovation'.
  
  **Patient-led Academy (EPF)**: Educate patient representatives and the lay public about all processes involved in medicines development.

- **ESO Master Class**

- **RCE – ESMO – ESO seminars**

- ....
Rare Disease Patient Engagement
Involved in EMA Committees

• **COMP**: Committee for Orphan Medicinal Products
  • 1 EUORDIS representative (Vice-Chair)
  • + 2 Observers

• **PDCO**: Paediatric Committee
  • 1 EUORDIS representative

• **CAT**: Committee for Advanced Therapies
  • 1 EUORDIS representative

• **PCWP**: Patients' and Consumers' Working Party
  • 2 EUORDIS representatives

• **Scientific Advice & Protocol Assistance**
Involved in EC Expert Groups, EU JAs & Projects, Conferences, EU Public Consultations

- **EUCERD/EC Expert Group on Rare Diseases (2011-2016):** 8 RD patient representatives (one for Rare Cancers) + 2 Observers (EURORDIS)
- **EC Expert Group on Cancer Control (June 2014 – June 2017):** 10 patient representatives including 2 EURORDIS’ representatives for Rare Cancers
- **European and international conferences (public health, research, social)**
- **EU Joint Actions (RD-ACTION, JARC, EUnetHTA) and other EU projects (health policies, research)**
- **Participation in EU Public Consultations:** responses shape EU policies, future Directive, Regulations
Patients & Industry Partnering in Innovation

Engagement of patient advocates all along the value chain of research & development of RD therapies as well as in access to medicines
ePAGs: The Voice of Patients in European Reference Networks (ERNs)

- As of early 2017, the EC and Board of EU MS endorsed the applications of 24 ERNs for complex diseases, requiring a high concentration of multidisciplinary medical expertise
- 4 ERNs are in the field of Rare Cancers (EURACAN, PaedCanERN, EuroBloodNet, GENTURIS)
- EURORDIS has established 24 European Patient Advocacy Groups (ePAGs), corresponding each to one ERN
- EURORDIS has organised elections and selections of ePAGs’ representatives based on their advocacy records and ability to represent all the patients of their ERN
- ePAGs’ representatives are part of ERN Boards/committees to provide the patients’ expertise
- Over 100 ePAGs’ representatives are supported in their work by EURORDIS
17 October 2017: Launch of the European Network of Parliamentary Advocates (MEPs) for Rare Diseases

A platform that:

- Discuss on specific challenges faced by people living with a rare disease
- Call for stronger EU-wide action
- Shape political input for future legislation and programmes
- Work to make RD an integral part of EU, national and regional programmes in health, research, social affairs and other relevant policies

A pool of advocates amongst policy makers:

- Selected MEPs - Members of the European Parliament
- National MPs to be identified and supported by National Alliances
Thank you for your attention

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Information on EURORDIS’ Advocacy achievements
Who are we?

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 750 rare disease patient organisations from more than 60 countries – including all 28 EU MS - that work together to improve the lives of the 30 million people living with a rare disease in Europe.

By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services.
EURORDIS in brief

• Founded in 1997
• 40+ staff, offices in Paris, Brussels, Barcelona
• Over 300 Volunteer patient advocates
• 6 € million budget
• 768 member patient organisations:
  711 members in Europe including 641 members in EU
• in 68 countries
  including all 28 EU MS + 13 other European countries
• Council of National Alliances of Rare Diseases
  33 member NAs
  2 CNA meetings / year
• Council of European Rare Disease Federations
  61 member European Federations of specific rare diseases,
  e.g. Cystic Fibrosis Europe, SMA Europe, CML…
  1 CEF meeting / year
What we do

Patient Empowerment

Advocacy

Information & Networking & Capacity Building

Research Policy

Access to Medicines, Therapies

Healthcare Policy & Services

Social Policy & Services
EURORDIS member patient organisations in Europe & beyond
EURORDIS Membership Meetings

A capacity building annual event for rare disease advocates


• An opportunity for EURORDIS Members to:
  • Join Capacity Building Workshops in priority policy areas
  • Network
  • Be updated in main policy and emerging issues
European Conference on Rare Diseases and Orphan Products (ECRD)

Organised by EURORDIS every two years


- Brings together all KEY stakeholders relevant in the field of rare diseases
- Provides the state of the art of the rare disease environment, monitoring and benchmarking initiatives in the field of research, improved healthcare pathways, innovative access to treatments, adapted social services
- ECRD 2016 in Edinburgh welcomed nearly 800 participants from 48 countries
- ECRD 2016 included 28 sessions with 120 speakers; 200 posters were displayed

www.rare-diseases.eu
Voicing rare disease patients’ expectations

Shaping EU Rare disease policies
Promote RDs in European Programmes

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

- New programmes
  - EU Health for Growth (2014-2020)
Major Advocacy Achievements
Contribution to EU medicines regulations

- EU Regulation on Orphan Medicinal Products in 1999
  - Creation of the COMP - Committee for Orphan Medicinal Products at the EMA (European Medicines Agency), including 3 patients’ representatives for the first time
  - 1868 Orphan Drugs Designated & 147 ODs approved by EC as of end August 2017 (benefiting ± 3 million EU patients)

- EU Regulation on Medicinal Products for Paediatric Use in 2006
  - Creation of the PDCO – Paediatric Committee at the EMA, including 3 patients’ representatives and their alternates

- EU Regulation on Advanced Therapy Medicinal Products in 2007
  - Creation of the CAT - Committee for Advanced Therapies at the EMA, including 2 patients’ representatives and their alternates
Involved in EMA Committees

- **COMP**: Committee for Orphan Medicinal Products
  - 1 EURORDIS representative (Vice-Chair)
  - + 2 Observers
- **PDCO**: Paediatric Committee
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- **PCWP**: Patients' and Consumers' Working Party
  - 2 EURORDIS representatives
- **Scientific Advice & Protocol Assistance**
Major Advocacy Achievements Shaping EU Rare Disease Policy

- **Communication from the European Commission** to the European Parliament, the Council, the European Economic and Social Committee and the Committee of the Regions: « Rare Diseases: Europe’s Challenge » 11 November 2008

- First comprehensive policy text addressing issues faced by RD patients, from research on RDs, to diagnosis, access to care and adapted services and development of training, education, awareness on RDs

- **Council Recommendation on an Action in the field of Rare Diseases, 8 June 2009**
  - Adopted by EU Ministers of Health
  - Promotes the adoption of RD National Plan/Strategy in EU MS
  - Creation of the EUCERD - European Union Committee of Experts on Rare Diseases (November 2009 – June 2013), replaced by the European Commission Expert Group on Rare Diseases (CEG-RD) (July 2013 – December 2013)
Advocacy Achievements Contribution to EU Directives/ Regulations

  - Provides the legal framework for the mobility of patients across EU
  - Article 12 on *European Reference Networks* for rare, complex conditions
  - Article 13 specifically on *Rare Diseases*

- **EU Regulation on Clinical Trials, 16 April 2014** (repealing EU Directive on Clinical Trials 2001/20/EC)
European Commission Expert Group on Rare Diseases (former EUCERD)

- Established in July 2013 until December 2016 (replacing EUCERD Nov. 2009-June 2013)
- Brings together European main decision-makers in the field of Rare Diseases
- Representation: 28 EU MS + Iceland, Norway, Switzerland + EC, EMA COMP + industry + academia + individual experts + patients’ representatives
- Objective: Assist the Commission in the drawing up of legal instruments and policy documents, including guidelines and recommendations, in the field of rare diseases.

All 8 patients’ representatives ➔ members of EURORDIS. They cover main rare disease patient support groups, including Rare Cancers, and different European regions. They coordinate advocacy work through regular contact.
Contribution to the Recommendations of EUCERD and GEG-RD

- **Quality Criteria for Centres of Expertise** for Rare Diseases in Member States (2011)
- **European Reference Networks for Rare Diseases** (2013) & Addendum (2015) related to regrouping rare diseases in broad clinical networks, and patient involvement in ERNs
- Support the **Incorporation of Rare Diseases Into Social Services and Policies** (2016)
- Ways to Improve **Codification** for Rare Diseases in Health Information Systems (2014)
- Rare disease patient registration and data collection (2013)
- Core indicators for rare disease national plans and strategies (2013)
- Improving **Informed Decisions Based on the Clinical Added Value of Orphan Medicinal Products** (CAVOMP) Information Flow (2012)

www.eurordis.org/content/new-eu-committee-experts-rare-diseases
Involved in CEG-CC - Commission Expert Group on Cancer Control

- Established in June 2014 until June 2017
- Brings together European main decision-makers in the field of Cancer
- **Objective**: Assist the Commission in drawing up legal instruments and policy documents, including guidelines and recommendations, in the field of cancers, **including rare cancers**
- **Representation**: 28 EU MS + Iceland, Norway, Switzerland + EC + industry + academia / cancer prevention + patients’ representatives
- 6 patients’ representatives ➔ Including 2 members of EURORDIS representing rare cancer patient support groups & different European regions. They coordinate advocacy work together with the EURORDIS group of volunteers on rare cancers.
European Joint Actions for Rare Diseases

- Jointly funded by the EC and EU MS
  - EUCERD Joint Action: March 2012-November 2015
  - RD-ACTION: June 2015-May 2018
- Support the activities of the EUCERD/CEG-RD to help shape EU RD policies:
  - Develop Recommendations for adoption by EUCERD/CEG-RD (e.g. European Reference Networks for Rare Diseases & Addendum)
  - Promote the adoption/implementation of national plans for RD in EU MS integrating EU Policies → 41 EUROPLAN national conferences & meetings co-organised by EURORDIS & National Alliances of Rare Diseases in Europe since 2010
  - Support Orphanet database & codification of RDs in health information systems
  - Integration of RDs into mainstream social policies (EURORDIS)
European Joint Action on Rare Cancers

- Jointly funded by the EC and EU MS
- JARC: October 2016 – September 2019
- Strong links with Joint Action RD-ACTION & the Commission Expert Groups on RDs and Cancer; notably on the shaping on European Reference Networks for RC
- Cover following areas:
  - Epidemiology
  - Quality Care Control
  - Good Practices Guidelines
  - Innovation
  - Medical Education
  - Childhood Cancers
  - Rare Cancers Policy
- EURORDIS is involved across all areas, together with ECPC (European Cancer Patients Coalition) and CCI-E (Childhood Cancer International - Europe) to input the patients’ perspective
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- **Call** for stronger EU-wide action
- **Shape** political input for future legislation and programmes
- **Work** to make RD an integral part of EU, national and regional programmes in health, research, social affairs and other relevant policies

A pool of advocates amongst policy makers:

- Selected **MEPs** - Members of the European Parliament
- **National MPs** to be identified and supported by National Alliances

EURORDIS.ORG
Voicing rare disease patients’ expectations

Fostering international research and policy initiatives
EURORDIS Involved in IRDiRC – International Rare Diseases Research Consortium

200 new therapies for rare diseases by 2020

Means to diagnose most rare diseases by 2020
International Rare Disease Research Consortium

- EURORDIS is a partner of the Consortium
- IRDiRC Consortium Assembly: 1 EURORDIS representative
- Therapies Scientific Committee: Yann Le Cam (Member) & Virginie Hivert (Vice-Chair)
- Task forces working with TSC:
  - Patient Relevant/Related Outcome Measures Task Force
  - Small Population Clinical Trials Task Force
  - Data Mining-Repurposing Task Force
  - One to come on ‘Patient Engagement in Research’
What is Rare Diseases International (RDI)?
RDI is the global alliance of people living with a rare disease of all nationalities across all rare diseases. It counts 40 member organisations at the end of 2016.

Who is involved?
RDI is a EURORDIS initiative set up with National Alliances around the world with whom we have signed partnership agreements (MoUs).

Why Rare Diseases International?
- To enhance capacities of Rare Diseases International members through information, exchange, networking, mutual support, joint actions
- To represent its members & people living with rare diseases internationally
- To promote RDs as an International Public Health & Research priority through public awareness and policy

www.rarediseasesinternational.org
What is the NGO Committee for Rare Diseases?
A substantive committee established under the umbrella of the Conference of NGOs in Consultative Relationship with the United Nations (CoNGO) to promote rare diseases as a priority in global health, research, and social and medical care as part of the UN 2030 Agenda: the Sustainable Development Goals (SDGs).

Who is involved?
It is an initiative by the Ågrenska Foundation and EURORDIS in cooperation with Rare Diseases International (RDI)

The formal inauguration of the Committee took place at the UN headquarters in New York on the 11th November 2016.

Video recording available at: www.ngocommitteerarediseases.org
A MILESTONE IN THE JOURNEY TO PUT RARE DISEASES IN THE GLOBAL AGENDA 2030

Founding Act “ Rare Diseases and the UN Sustainable Development Goals ”
New York City, Friday 11 November 2016

We, the undersigned participants to the inaugural “Global Gathering for Rare Diseases”, proclaim and endorse the following statements:

☐ Each of the 6,000 repertoried rare diseases affects a very small population locally. All together, however, they represent an international public health issue.

☐ The 350 million persons living with a rare disease worldwide are facing common challenges across diseases and across borders that affect all parts of their lives at once.

☐ No one country, no one continent alone can solve the problems posed by rare diseases

☐ Experience shows that actions are possible and effective. Common national policies and international collaboration can address these challenges.

☐ Scientific, medical, technological, social opportunities will be high in the next 20 years. Patients, NGOs and other stakeholders are committed and aligned for partnership.

☐ Rare diseases fit within the objectives from several UN SDGs and can significantly contribute to their achievement.

The NGO Committee for Rare Diseases will catalyse all efforts towards the delivery of the SDGs in support of rare diseases, and towards the recognition and integration of rare diseases in all relevant future global policy and initiatives of the United Nations and its agencies.

[Signatures]

[Logo: NGO Committee for Rare Diseases]
Creating value for people living with a rare disease

- Catalyse transformative change
- Provide a cohesive voice
- Act as a knowledge broker
- Empower people living with a rare disease
- Elevate the patient perspective
- Connect people living with a rare disease

EURORDIS

European Conference on Rare Diseases & Orphan Products

EURORDIS ROUND TABLE OF COMPANIES