Global Strategies for Rare Diseases: The Patients' perspective

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EURORDIS in brief

- Founded in 1997
- 692 member patient organisations
- 63 countries (more than half from outside Europe)
- 38 National Alliances of RD Patients Organisations
- 51 European Federations of specific rare diseases
- Outreach to over 1800 patient groups
- 30+ staff, offices in Paris, Brussels, London, Barcelona, Geneva
- Over 320 Volunteers: +80 patient advocates and +250 moderators
- 5 Million € Budget

EURORDIS Board of Directors

EURORDIS Staff

Rarity calls for action at global level

- Rare Disease expertise is scarce and scattered
- Need to bring together a critical mass of :
  - patients and medical experts
  - scientists and public health authorities

- Need to support countries emerging to rare diseases
- Need to work with companies acting at global level

Need for more Rare Diseases & Orphan Drugs Regulations

- **1983**: USA: The Orphan Drug Act (ODA) →promoted by the National Organization for Rare Disorders (NORD)
- **1991**: SINGAPORE
- **1993**: JAPAN
- **1997**: AUSTRALIA
- **1999**: EU Regulation on Orphan Drugs →EURORDIS was at the forefront of this Regulation
- Other parts of the world lagging behind

Challenges & Hopes

- 6000-7000 different rare diseases
- Differ greatly from each other, great heterogeneity, affect different organs, different disabilities, different age groups
- Different health systems, gaps in economic and social development across the world North/South divide

- However there are **commonalities across all rare diseases**. They share many of the **same challenges and issues in all parts of the world** which allows for common healthcare policies

- In the 21st century, new opportunities from translational research and information technologies to:
  - Bring more innovative treatments to patients
  - Access information
  - Create networking opportunities globally

The globalisation of RD is underway

- Development of National Plans / Strategies worldwide
- Increased investments in RD Research : IRDiRC, increased budget of the US NIH or EU dedicated to RD Research…
- Increasing international networking amongst scientists (ReACT Congress + e-Rare)
- Increased investments from industry with market exclusivity incentives provided in several legislations (US Orphan Drug Act, EU Regulation (EC) 141/2000, ….)
- Increased international collaboration amongst regulatory agencies: EMA & FDA; COFEPRIS & other LatAm countries
- EU & WHO Data Sharing Pact on the safety, quality and efficacy of medicines that are already authorised or under review
- Development of international platforms for RD registries
- Rare disease patients getting organised internationally

EURORDIS & INTERNATIONAL

- *Rare Disease Day*
  - Last day of February
  - 18 European countries in 2008
  - 80+ countries worldwide in 2015
  - Patient organisations from Bangladesh, Bolivia & Madagascar
- *RareConnect*
  - 75+ global online patient communities
  - 661 patient groups
  - free translations between 6 languages English, French, German, Italian, Spanish, Portuguese (new)
- *European Conference on Rare Diseases & Orphan Products (ECRD)*
  - 8th edition in May 2016, Edinburgh, Scotland
  - Attract +700 partcipants from around the world
  - Translations in French, Spanish, German, Italian and Russian

EURORDIS & INTERNATIONAL

- EURORDIS Round Table of Companies
  - +45 multinational pharma and biotech companies
- International Rare Disease Research Consortium (IRDiRC)
  - EURORDIS is a partner
  - EURORDIS 1 of 3 patient reps at ExeCo
  - Therapies Scientific Committee: Yann Le Cam, Elected Chair
- Partnerships with National Alliances and International Federations
  - Council of National Alliances (25)
  - Council of European / International Federations (31)
  - MoUs with NORD (US), CORD (Canada), JPA (Japan), RVA (Australia), RPU (Russia)
- Rare Diseases International

RDI

RARE DISEASES INTERNATIONAL

A EURORDIS INITIATIVE

- What is Rare Diseases International (RDI)?

  An initiative to create the global alliance representing patients & families of all nationalities across all rare diseases.

- Where does it come from?

  RDI is a EURORDIS initiative with National Alliances around the world with whom we have signed partnership agreements and with whom we have the same international vision

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

RDI LAUNCH EVENT
MAY 28, 2015
MADRID, SPAIN

GOVERNANCE

RARE DISEASES INTERNATIONAL

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The preliminary phase of the initiative

has been steered by a Pre-Formation Group

1. Europe (EURORDIS)
2. USA (NORD)
3. Canada (CORD)
4. Japan (JPA)
5. China (CORD)
6. India (I-ORD)
7. Ibero-American pan-regional alliance (ALIBER)
8. DEBRA International – International Federation for Epidermolysis Bullosa
9. IPOPI - International Patient Organisation of Primary Immunodeficiencies

25 MEMBERS SO FAR

EURORDIS
ALIBER-Alianza Iberoamericana de Enfermedades Raras
ALLIANCE MALADIES RARES
Arabic Organisation for RareDiseases
Associacao Brasileira de Enfermedades Raras
CANADIAN ORGANIZATION FOR RARE DISORDERS
Chinese Organization for Rare Disorders
Cyprus Alliance for Rare Disorders
Debra International
Federación Argentina de Enfermedades Poco Frecuentes
FEDERACIÓN ESPAÑOLA DE ENFERMEDADES RARAS
Indian Organization for Rare Diseases
IPOPI - INTERNATIONAL PATIENT ORGANIZATION FOR PRIMARY IMMUNODEFICIENCIES
Japan Patient Association
MALAYSIAN RARE DISORDERS SOCIETY
NEW ZEALAND ORGANISATION FOR RARE DISORDERS
NORD NATIONAL ORGANIZATION FOR RARE DISORDERS
Pro Rare Austria, Allianz für seltenen Erkrankungen
Rare Disease Society of South Africa
Rare Voices Australia Ltd
Retina International
Hongkong Alliance for RD
GENETIC ALLIANCE AUSTRALIA
PH LATIN SOCIETY
BLACKSWAN FOUNDATION

ADVOCACY

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- Promote the Joint Declaration "Rare Diseases: an International Public Health Priority" – 10 recommendations
  - Enhanced **Visibility**
  - Importance of **Patient Empowerment**
  - The right to **Universal Access to Healthcare**
  - International coordination of **Research**
  - **Shaping Policy** at international level
- Introduce concept and foster understanding of rare diseases in international institutions (UN, ECOSOC, WHO, PAHO, OECD)
- UN Committee for Rare Diseases (through the Conference of NGOs with Consultative Status to the United Nations' Economic and Social Council)

NETWORKING

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- Encourage the formation and supporting existing National Alliances of RD patients
- Encouraging the formation and enlargement of rare disease-specific federations
- Encourage and facilitate regional networking of rare disease patient organisations e.g. IberoAmerican Alliance; Asia Pacific Alliance, etc
- Short-Term Exchange & Internship Capacity Building Programme between member organisations

BUILDING BRIDGES WITH OTHER STAKEHOLDERS

Bring all stakeholders together to build a solid ecosystem at the international level:

- Rare Diseases International
- International Conferences for Rare Diseases and Orphan Drugs (ICORD)
- International Alliance of Patients' Organizations (IAPO)
- International Federation of Pharmaceutical Manufacturers & Associations (IFPMA)
- International Rare Diseases Research Consortium (IRDiRC)
- International Society of Human Genetics (ISHG)
- Orphanet International (10 countries ex Europe)

ICORD and RDI Collaboration

- ICORD and RDI have signed a formal agreement announced publicly today
- The collaboration is based on the key common strategic objective to promote rare diseases as an international public health priority
- Will focus on **partnering for the development and organisation of the annual conference**
- Aim to exploit common synergies and streamline efforts
- The next ICORD in October 2016 in South Africa will be the first opportunity to showcase this collaboration

EURORDIS Rare Diseases Europe

THANK YOU

ICORD
International Conference on Rare Diseases & Orphan Drugs

EURORDIS
Rare Diseases Europe

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