ACHIEVEMENTS OVER THE PAST 20 YEARS AND FUTURE GOALS IN ADVOCACY

Yann Le Cam
EURORDIS

19 May 2017, EURORDIS Membership Meeting, Budapest
Rare diseases now recognised as a public health priority

Since 1999, the European Union has first recognised rare diseases as a public health priority: EU Orphan Product Regulation.

- **1999**: EU Paediatric Regulation
- **2006**: EU Advanced Therapies Regulation
- **2008**: European Commission Communication on Rare Diseases
- **2009**: European Council Recommendation on an action in the field of Rare Diseases
- **2011**: EU Directive on patients’ rights in cross-border healthcare
A strong European Patient Voice

58 European Federations in 2017 (from 25 Federations in 2010)

34 National Alliances of Rare Disease Patient Organisations in 32 countries (from 23 National Alliances in 2010)

From 187 patient organisations in 2000 to over 700 in 2017
New medicines for rare diseases

1999 EU Regulation on Orphan Medicinal Products introduced incentives for companies looking to develop orphan medicines

Over 1800 medicines have received an orphan designation

126 medicines have been approved for rare diseases

Orphan drug designations
Marketing authorisations of orphan drugs and non-orphan drugs
24 European Reference Networks

PATIENTS  →  NATIONAL HEALTHCARE PROVIDERS  →  SPECIFIC ERN

TREATMENT  ADVICE

CLINICAL GUIDELINES
RESEARCH & INNOVATION KNOWLEDGE
GENERATING & SHARING EVIDENCE
TRAINING & E-LEARNING

▷ 300 HOSPITALS

THOUSANDS OF PATIENTS HELPED BY 2020

▷ 900 HEALTHCARE UNITS
Participation & Responsibilities

European Reference Networks (ERNs)

- ERN BOND - European Reference Network on bone disorders
- ERN CRANIO - European Reference Network on craniofacial anomalies and ear, nose and throat (ENT) disorders
- Endo-ERN - European Reference Network on endocrine conditions
- ERN EpiCARE - European Reference Network on epilepsies
- ERKNet - European Reference Network on kidney diseases
- ERN-RND - European Reference Network on neurological diseases
- ERN-ICICA - European Reference Network on inherited and congenital anomalies
- ERN LUNG - European Reference Network on respiratory diseases
- ERN Skin - European Reference Network on rare and undiagnosed skin disorders
- ERN EURACAN - European Reference Network on haematological diseases
- ERN eUROGEN - European Reference Network on urogenital diseases and conditions
- ERN EURO-NMD - European Reference Network on neuromuscular diseases
- ERN EYE - European Reference Network on eye diseases
- ERN GENTURIS - European Reference Network on genetic tumour risk syndromes
- ERN GUARD-HEART - European Reference Network on diseases of the heart
- ERN ITHACA - European Reference Network on congenital malformations and rare intellectual disability
- MetabERN - European Reference Network on hereditary metabolic disorders
- ERN PaedCan - European Reference Network on paediatric cancer (haemat-oncology)
- ERN RARE-LIVER - European Reference Network on hepatological diseases
- ERN ReCONNET - European Reference Network on connective tissue and musculoskeletal diseases
- ERN RITA - European Reference Network on immunodeficiency, autoinflammatory and autoimmune diseases
- ERN TRANSPLANT-CHILD - European Reference Network on Transplantation in Children
- VASCERN - European Reference Network on Rare Multisystemic Vascular Diseases
**EURORDIS’ REPRESENTATION, PARTNERSHIP AND SUPPORT TO NETWORKS AND ORGANISATIONS IN 2017**

**European Not-for-Profit Organisations:**
- DIA: Drug Information Association
- EFPIA Think Tank: European Federation of Pharmaceutical Industries and Associations
- EUROPABIO Patients Advisory Group
- EUCOPE
- EPF: European Patients’ Forum
- EFGCP: European Forum for Good Clinical Practice
- FIPRA – International Policy Advisors
- Friends of Europe
- Rare Cancer Europe
- Social Platform
- Maladies Rares Info Service (French Helpline for RDs)
- Rare Disease Platform in Paris
- PFMD - Patient Focused Medicines Development Initiative

**International Institutions and Not-for-Profit Organisations:**
- NGO Committee for Rare Diseases (United Nations, New York)
- NEWDIGS: New Drug Development Paradigms
- IAPO: International Alliance of Patients’ Organizations
- IRDiRC: International Rare Disease Research Consortium
- ICORD: International Conference on Rare Diseases and Orphan Drugs

**Member of European Networks:**
- E-Rare
- EuroBioBank
- ECRIN
- BBMRI Stakeholders Forum
- Treat NMD
- RD-Connect
- SCOPE Joint Action (Advisory Board)
- OpenMedicine
- IMI EUPATI
- IMI ADAPT-SMART

**Partnership Learned Societies:**
- European Federation of Internal Medicine (EFIM)
- European Hospital & Healthcare Federation (HOPE)
- International Federation of Social Workers Europe (IFSW-Europe)
- European Society of Human Genetics (ESHG)
- International Society for Pharmaco-economics and Outcomes Research (ISPOR)

**European network of parliamentarian advocate for rare diseases:**
- European parliament interest group on Rare Diseases
- Advocates in national parliaments
Rare Diseases International
A strong international voice
NGO Committee for Rare Diseases

• 11th November 2016 inauguration of the Committee at the UN headquarters in New York
• Established to promote rare diseases as a priority in global health, research, and social and medical care as part of the UN 2030 Agenda
Report on impact of Council Recommendation Rare Diseases, 2019

European Parliamentarian Advocate Network launch

2017

ERN launch

2018

European Joint Programme for Rare Disease Research - 2019 – 2030

2019

Foresight Rare 2030

2020

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UN Resolution on Rare Diseases?

New Council Recommendations?