



# ACHIEVEMENTS OVER THE PAST 20 YEARS AND FUTURE GOALS IN ADVOCACY

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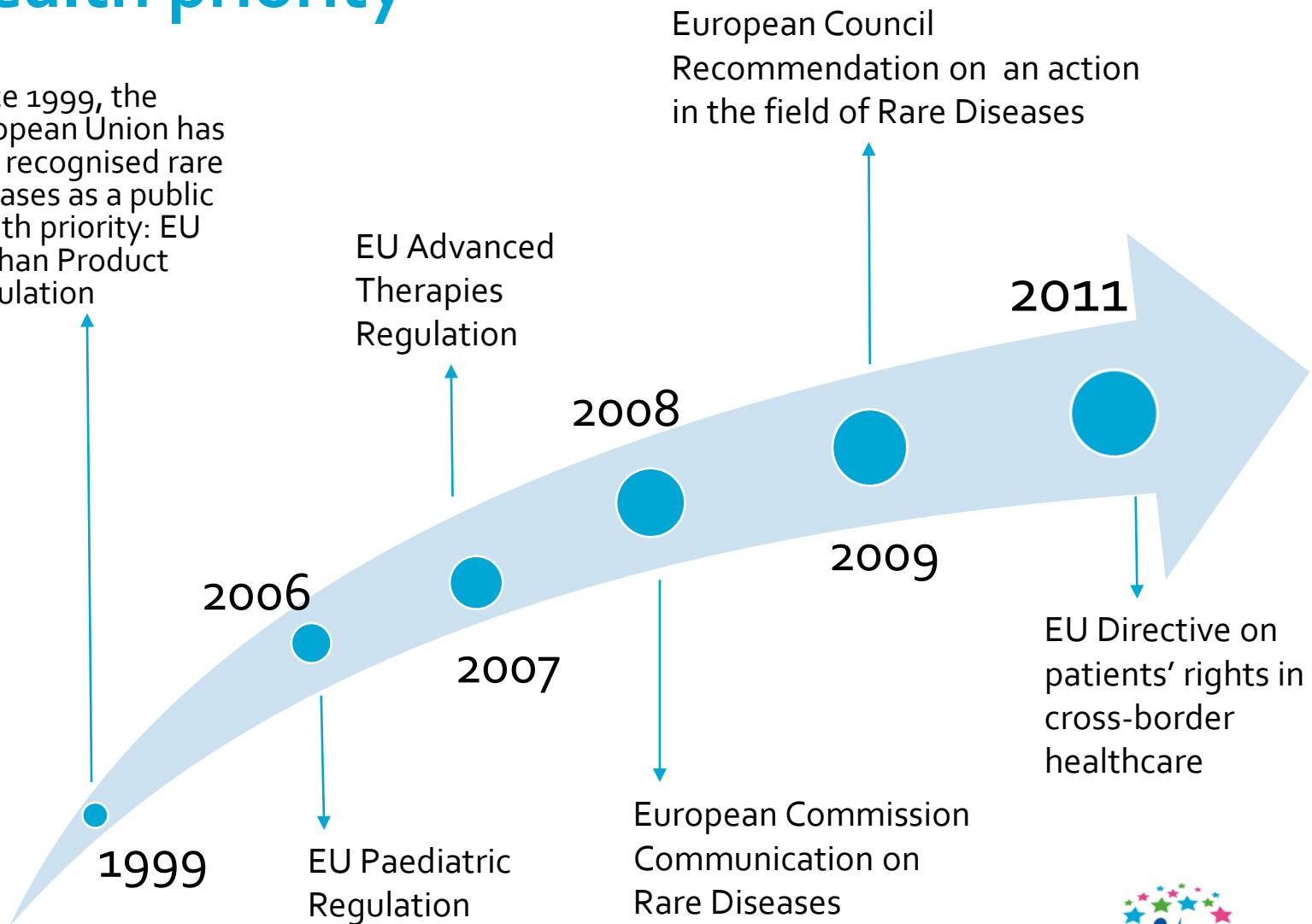
19 May 2017, EURORDIS Membership Meeting, Budapest

[EURORDIS.ORG](http://EURORDIS.ORG)



# 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



# A strong European Patient Voice

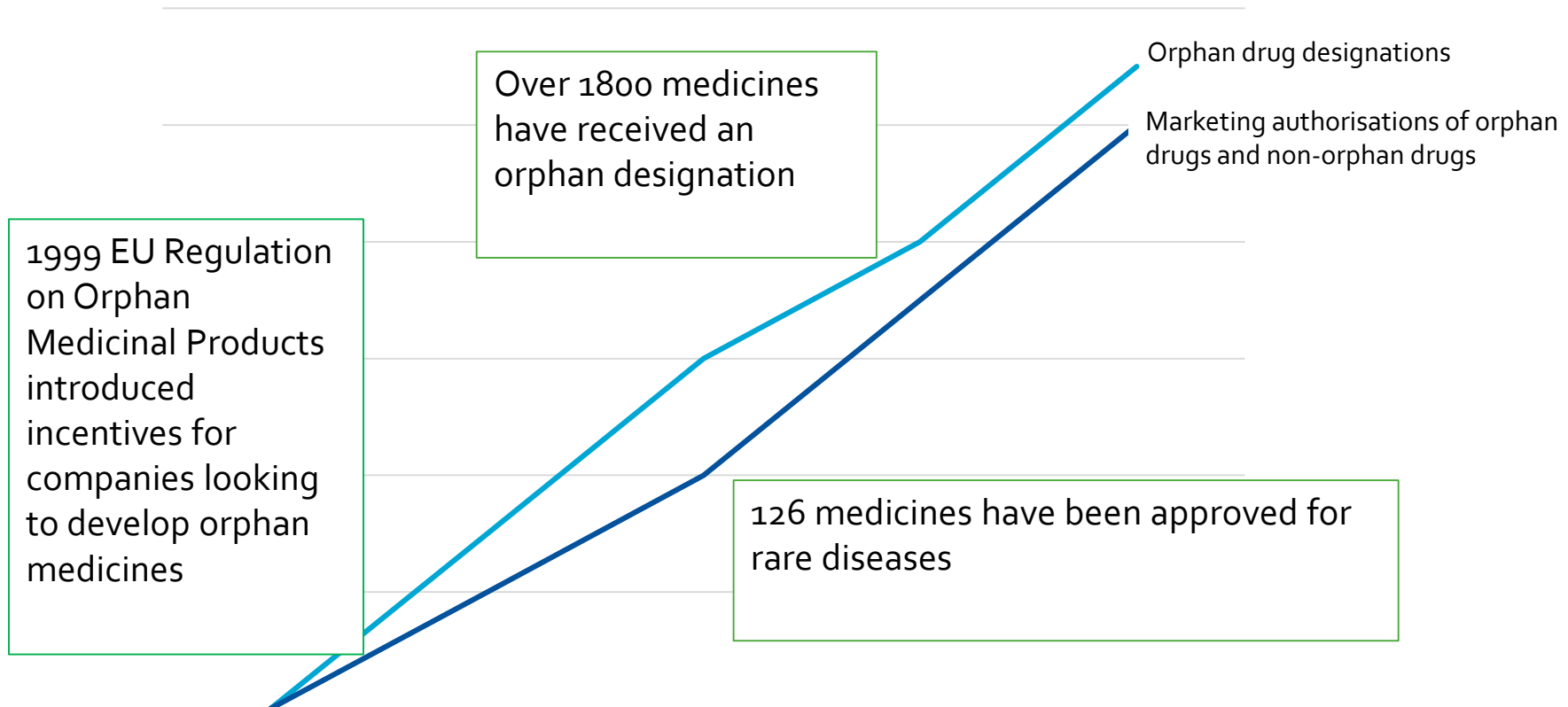
From **187 patient organisations** in 2000 to over 700 in 2017

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

**EURORDIS' growth**

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

# New medicines for rare diseases



# 24 European Reference Networks



> **300**  
**HOSPITALS**

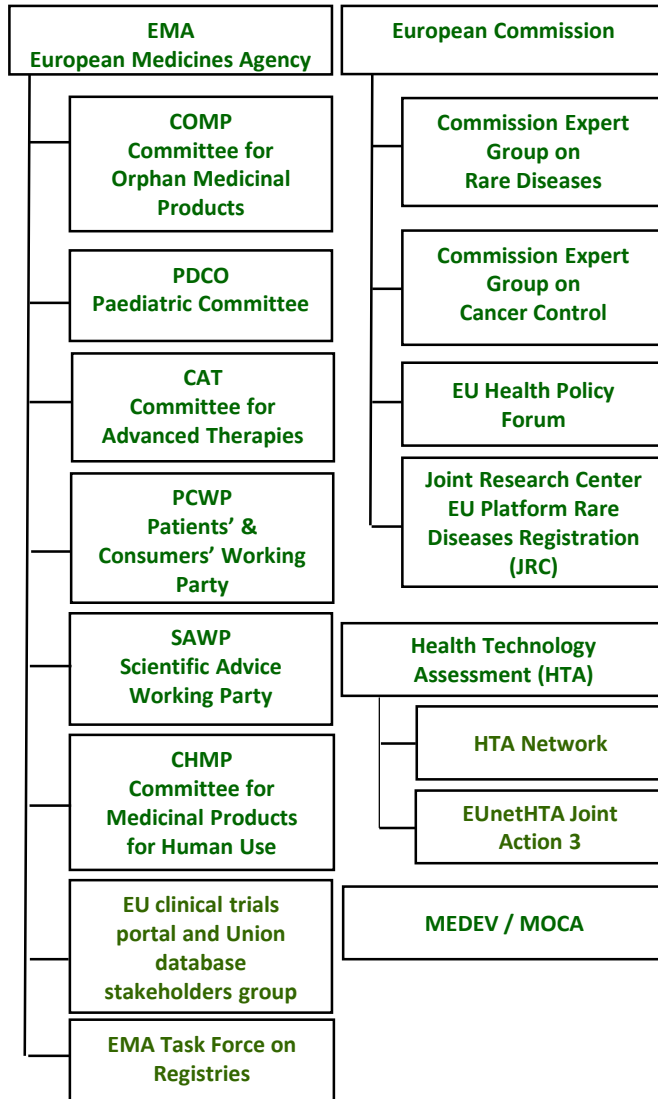


**THOUSANDS OF**  
**PATIENTS HELPED**  
**BY 2020**



> **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
- **ERNICA** - 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 adult cancers (solid tumours)
- **ERN EuroBloodNet** - 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 (haemato-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

### European network of parliamentarian advocate for rare diseases:

European parliament interest group on Rare Diseases

Advocates in national parliaments

### 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 Pharmacoeconomics and Outcomes Research (ISPOR)



# Rare Diseases International

## A strong international voice



RARE  
DISEASES  
INTERNATIONAL

A EURORDIS INITIATIVE







NGO COMMITTEE FOR  
RARE DISEASES

# NGO Committee for Rare Diseases

- 11<sup>th</sup> 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



European Parliamentary Advocate Network launch

Report on impact of Council Recommendation Rare Diseases, 2019 ?

202X



202y

2019



2020

Foresight Rare 2030

UN Resolution on Rare Diseases ?



2018

European Joint Programme for Rare Disease Research - 2019 – 2030

New Council Recommendations ?

2017



ERN launch