EURORDIS
RARE DISEASES EUROPE

EURORDIS-Rare Diseases Europe

The Voice of Rare Disease Patients in Europe

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Senior Manager of Relations with Patient Organisations

Lupus Europe meeting, 28 November 2020

EURORDIS.ORG

Rare diseases

OVER
6000
distinct rare diseases

Each one affects fewer than
1 IN
2000
PEOPLE

All together, an estimated
30
MILLION PEOPLE
are living with a rare disease in Europe and
300
MILLION
worldwide

72%
of rare diseases are genetic

Affects between
3.5%
-
5.9%

of the population in the course of their lives

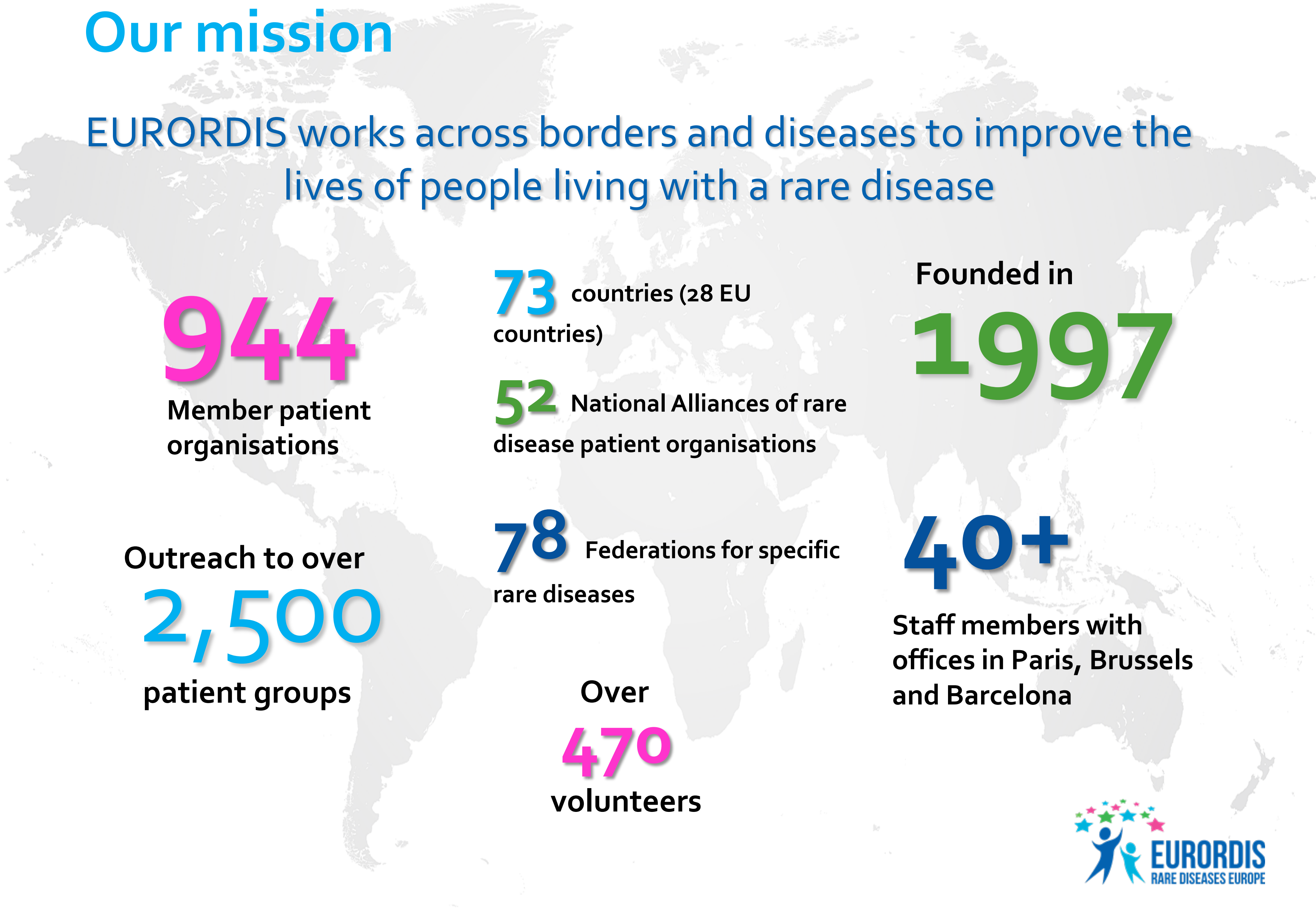
NO
CURE
for the vast majority of diseases and few treatments available

Onset of
70%
of rare diseases is in childhood

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.

Daniel, Williams syndrome

Our mission



EURORDIS works across borders and diseases to improve the lives of people living with a rare disease

944
Member patient organisations

73 countries (28 EU countries)

52 National Alliances of rare disease patient organisations

Founded in
1997

Outreach to over
2,500
patient groups

78 Federations for specific rare diseases

40+
Staff members with offices in Paris, Brussels and Barcelona

Over
470
volunteers

EURORDIS
RARE DISEASES EUROPE

EURORDIS Members for Lupus & Scleroderma & Sjögren

Lupus	Scleroderma	Sjogren
Association Française du Lupus et autres Maladies Auto-Immunes	Asociación de Esclerodermia Castellon	Asociación Española Sindrome De Sjögren
Lupus France	Asociación Española de Esclerodermia	Assocciazione Persone con Malattie Reumatiche
Association Lupus Erythematosus	Assocciazione Persone con Malattie Reumatiche	Association Française du Gougerot-Sjögren
	Gruppo Italiano per la lotta alla Sclerodermia	Liga Portuguesa contra as Doenças Reumáticas
	National Association ReumaZorg Nederland	
	Sklerodermie Selbsthilfe e.V.	
Lupus Europe	**FESCA**	**Sjögren Europe**

ADVOCATING FOR PATIENTS

EMPOWERING PATIENTS

EURORDIS
RARE DISEASES EUROPE

ENGAGING PATIENTS

PATIENTS FIRST

ADVOCATING FOR PATIENTS

Iris, Ehlers-Danlos syndrome, chronic paroxysmal hemicrania, cluster headaches

EURORDIS advocates 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

European Patient Advocacy Group

ecrd

914027

IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

EUROPEAN JOINT PROGRAMME RARE DISEASES

RARE DISEASES INTERNATIONAL

NGO COMMITTEE FOR RARE DISEASES

Rare 2030
Foresight in Rare Disease Policy

Our advocacy is fuelled by **real-life experiences of the rare disease community** through the EURORDIS Rare Barometer survey programme

Rare Barometer
A EURORDIS INITIATIVE

Major Advocacy Achievements

- **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

- **Over 1500 Orphan Drugs Designated** & more than 100 Products approved, benefiting approximately 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

Major Advocacy Achievements Shaping EU Rare Disease Policy

- Communication from the European Commission to the European Parliament, : « 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 (**Promote the adoption of RD National Plan/Strategy in EU MS)**

- EU Directive on Patients' Rights in Cross-border Healthcare, 9 March 2011

- EU Regulation on Clinical Trials, 16 April 2014

- EU "Data Protection Package", 27 April 2016

Rare Barometer, a EURORDIS initiative

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

Survey on the impact of Covid19 on rare disease patients – Main features

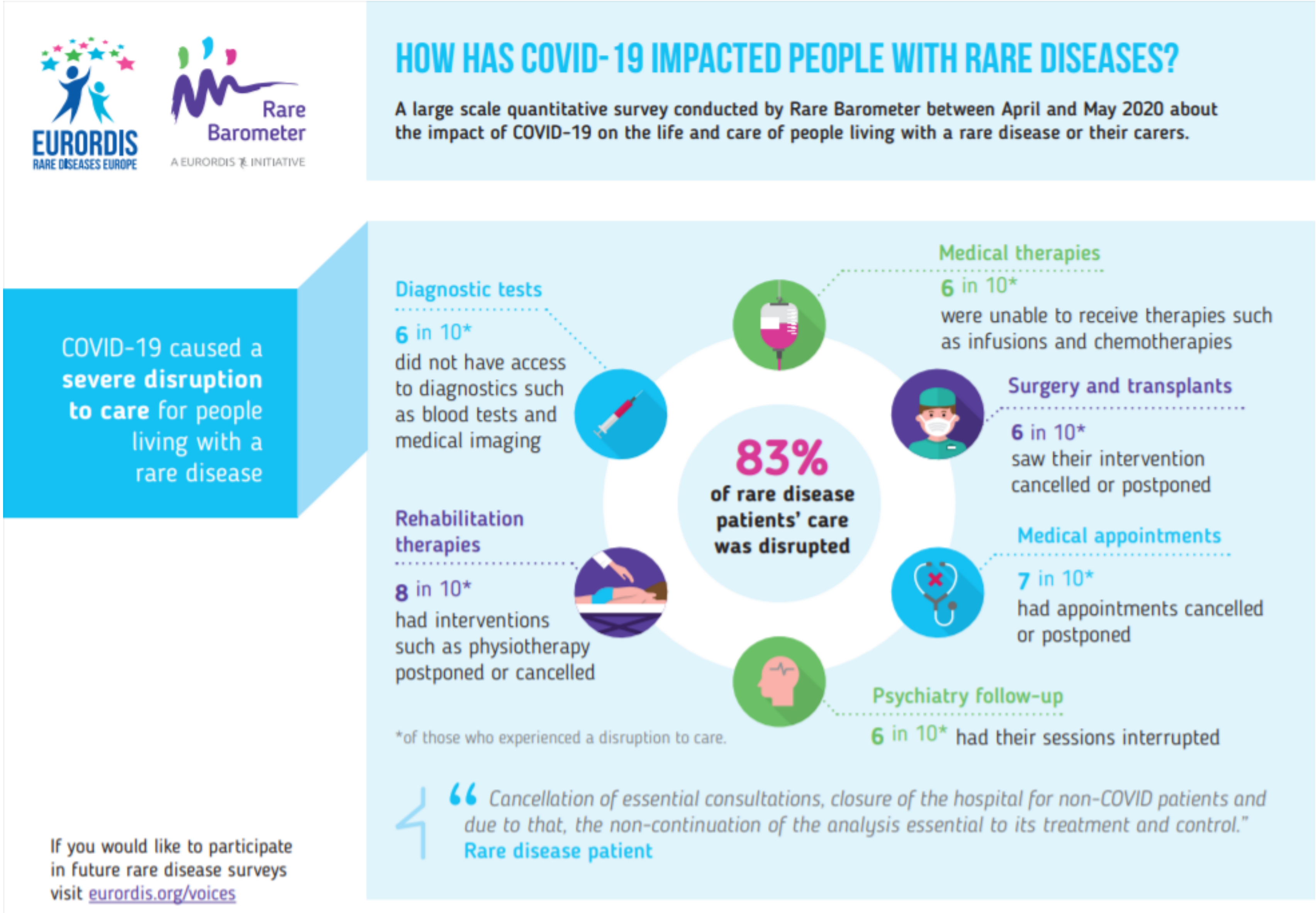
18th April – 11th May

6945 respondents in Europe (RD patients and family members)

Online quantitative survey, disseminated through social media and through EURORDIS’ patient organisation network

Translated in **23 languages**

Exemple :Interruption of care



HOW HAS COVID-19 IMPACTED PEOPLE WITH RARE DISEASES?

A large scale quantitative survey conducted by Rare Barometer between April and May 2020 about the impact of COVID-19 on the life and care of people living with a rare disease or their carers.

COVID-19 caused a **severe disruption to care** for people living with a rare disease

83% **of rare disease patients' care was disrupted**

Diagnostic tests
6 in 10*
did not have access to diagnostics such as blood tests and medical imaging

Medical therapies
6 in 10*
were unable to receive therapies such as infusions and chemotherapies

Surgery and transplants
6 in 10*
saw their intervention cancelled or postponed

Rehabilitation therapies
8 in 10*
had interventions such as physiotherapy postponed or cancelled

Medical appointments
7 in 10*
had appointments cancelled or postponed

Psychiatry follow-up
6 in 10* had their sessions interrupted

*of those who experienced a disruption to care.

> *Cancellation of essential consultations, closure of the hospital for non-COVID patients and due to that, the non-continuation of the analysis essential to its treatment and control."*
> **Rare disease patient**

If you would like to participate in future rare disease surveys visit eurordis.org/voices

Participate in Rare Barometer surveys and make your voice heard

https://www.eurordis.org/voices

Upcoming studies

Nov - Jan 2020 - The future of your rare disease. Early 2021 - Rare disease diagnosis

Use the results of former studies

http://download2.eurordis.org/rbv/covid19survey/covid_infographics_final.pdf

For results for your disease, please contact:

rare.barometer@eurordis.org

EMPOWERING PATIENTS

Kacper from Poland is living with Kawasaki disease

EURORDIS empowers 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

EURORDIS OPEN ACADEMY

EURORDIS Membership Meeting

RAREDISEASEDAY.ORG

RareConnect.org

eNews: 19.09.19

Donate

EURORDIS RARE DISEASES EUROPE

The Voice of Rare Disease Patients in Europe

From concept to reality: true patient partnership in European Reference Networks

The European Reference Networks (ERNs) connect healthcare providers across Europe in virtual networks to provide the best treatment options for people living with a rare disease. It is vital that patients are empowered partners in the development and governance of these networks. To this end, EURORDIS created the European Patient Advocacy Groups (ePAGs). There are now over 250 ePAG Advocates connecting the ERNs to the wider rare disease patient community in Europe. Caroline is one of them. Read her story.

Read

European Network of Rare Disease Help Lines

EURORDIS
OPEN ACADEMY

EURORDIS Open Academy

Current Training Offer

Blended learning

Face-to-face training

Online sessions & Webinars

e-Learning

EURORDIS
WINTER SCHOOL
on Scientific Innovation & Translational Research
30 patient advocates

EURORDIS
SUMMER SCHOOL
on Medicines Research & Development
30 patient advocates & 10 researchers

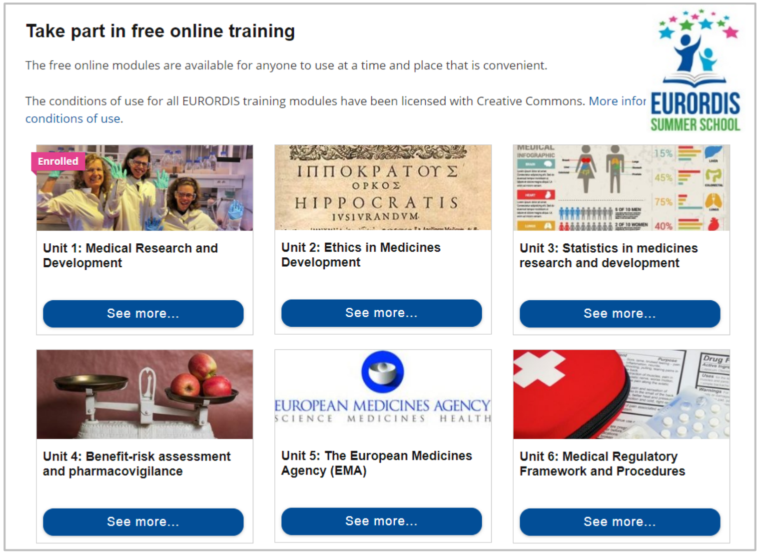
EURORDIS
DIGITAL SCHOOL
on Social & Digital Media
Online, open to all RD patient advocates

EURORDIS
LEADERSHIP SCHOOL
on Healthcare & Research
30 ePAG Advocates - European Patient Advocacy Groups

All trainings are in English

e-Learning: 50+ online courses

https://openacademy.eurordis.org



Take part in free online training

The free online modules are available for anyone to use at a time and place that is convenient.

The conditions of use for all EURORDIS training modules have been licensed with Creative Commons. More infor conditions of use.

Unit 1: Medical Research and Development

Unit 2: Ethics in Medicines Development

Unit 3: Statistics in medicines research and development

Unit 4: Benefit-risk assessment and pharmacovigilance

Unit 5: The European Medicines Agency (EMA)

Unit 6: Medical Regulatory Framework and Procedures

Take part in free online training

Learn more about rare disease research at a time and place that suits you! Take the free online modules bel includes a lesson and quiz.

The below online modules are from the first edition of the EURORDIS Winter School. Full online modules wil following the 2019 edition of the EURORDIS Winter School. The conditions of use for all EURORDIS training h with Creative Commons.

Winter School Unit 1: Setting the landscape

Winter School Unit 2: Genetic research to clinical diagnosis of Rare Diseases

Winter School Unit 3: Sharing patient data

Winter School Unit 4: From research to therapies

Free Open Access

ENGAGING PATIENTS

Philipp, osteogenesis imperfecta

EURORDIS engages 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.

Healthcare

European Reference Networks

Medicines life cycle

Research

European Patient Advocacy Group

PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EURORDIS EUROCAB Programme

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS 4 CHILDREN

EUROPEAN JOINT PROGRAMME RARE DISEASES

IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

Solve RD
Solving the Unsolved Rare Diseases

The EURORDIS **Council of National Alliances** (CNA) is made up of **30+ national rare disease alliances** in Europe that come together to share information & best practices

The EURORDIS **Council of European Rare Disease Federations** (CEF) is a platform for exchange across **70+ federations** working for a disease or group of diseases.

EURORDIS volunteers at the heart of policy making

440+ volunteers involved in EURORDIS activities

Drug Information, Transparency and Access Task Force (DITA)
focusses on the areas of product information, transparency of the regulatory process and access to medicines

30 + ePAG Advocates who are members of the EURORDIS **ePAG Steering Committee** active across 24 European Reference Networks

RARE DISEASE DAY®

Rare Disease Day Working Group
Co-creating the global campaign

HTA Task Force
to facilitate the participation of patients in health technology assessment activities

340+ **RareConnect moderators** animate the RareConnect communities

SPAG (Social Policy Action Group) advocating for holistic & integrated care

Therapeutic Action Group (TAG), composed of EURORDIS volunteers in the scientific committees and working party at the **European Medicines Agency** (EMA)

ePAGs

302 ePAG advocates

from 263 patient organsiation

of which 189 are EURORDIS members

In 29 countries

ePAG: ERN ReCONNET (Connective tissue and musculoskeletal)

7 ePAG patient representatives:

- Belgium, Alain Cornet, Lupus Europe
- Belgium, Charissa Frank, Vlaamse Vereniging voor erfelijke Bindweefselaandoeningen
- Germany, Juergen Grunert, Deutsche Ehlers-Danlos Initiative e.V.
- Italy, Ilaria Galetti, Federation of European Scleroderma Associations
- Portugal, Ana Vieira, Liga Portuguesa contra as Doenças Reumáticas
- Portugal, Vera Guimaraes, Liga Portuguesa contra as Doenças Reumáticas
- Spain, Silvia Aguilera, , Asociación Española Del Síndrome De Rett