EURORDIS
RARE DISEASES EUROPE

EURORDIS.ORG

ACTIVITY REPORT 2024

Cover photo: “A little guest (Uncle’s wedding day)”
Infantile free sialic acid storage disease (ISSD) - Croatia

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FOREWORD

AVRIL DALY
President of the Board of Officers

VIRGINIE BROS-FACER
Chief Executive Officer

As we reflect on the remarkable journey of EURORDIS-Rare Diseases Europe in 2024, we are filled with a sense of pride and optimism for the future of rare disease advocacy and care. This year has been marked by significant strides in our mission to improve the lives of those impacted by rare conditions across Europe. While these global challenges presented obstacles, they also underscored the importance of international cooperation in addressing rare conditions.

Strengthening Our Collective Voice

The power of our organisation lies in the strength of our network, and 2024 saw unprecedented growth in this regard. With our membership expanding to encompass over 1,000 organisations, we have amplified the voices of rare disease patients and their families like never before. This growth has not only increased our reach but has also enriched our understanding of the diverse challenges faced by the rare disease community.

Our Council of National Alliances (CNA) has been instrumental in coordinating efforts across Europe, ensuring that the needs of rare disease patients are addressed at both national and EU levels. This collaborative approach has been crucial in pushing for more comprehensive and inclusive health policies. The CNA has ensured that rare disease advocacy remained a unifying force, transcending political boundaries.

EURORDIS continued to promote rare conditions as an international public health priority. Collaboration with Rare Diseases International (RDI) in 2024 led to a significant milestone in February 2025 with the official recommendation by the WHO Executive Board to the 78th World Health Assembly, which will take place in May 2025 with the adoption of the Resolution on Rare Diseases. This accomplishment demonstrates the power of united advocacy in overcoming barriers to improve rare disease care globally.

Pioneering Research and Policy Initiatives

The conclusion of the European Joint Programme on Rare Diseases and the launch of ERDERA marked a new era in rare disease research. These initiatives have created a robust ecosystem that bridges the gap between research, clinical care, and innovation, promising to accelerate the development of new treatments and diagnostic tools.

Our advocacy efforts have been relentless, with a particular focus on the need for a European Action Plan for Rare Diseases. By engaging with key stakeholders at all levels of European governance, we have kept rare conditions at the forefront of health policy discussions in Europe, paving the way for more targeted and effective interventions.

Empowering Patients and Advancing Care

Education and patient empowerment remain at the heart of our mission. The expansion of our Open Academy and the launch of EUCAPA demonstrate our commitment to equipping patients and

their representatives with the knowledge and skills needed to actively participate in healthcare decision-making processes.

Our Rare Barometer surveys have provided invaluable insights into the challenges faced by people living with rare conditions, from diagnostic odysseys to the impact on daily life. These findings are not just statistics; they are powerful tools that inform policy and drive change based on evidence gathered from the rare disease patient community.

Innovating for Better Treatment Access

In the realm of treatment development and access, EURORDIS has been at the forefront of discussions on the revision of the General Pharmaceutical Legislation. Our involvement in projects like REMEDi4ALL and JOIN4ATMP is helping to pave the way for more accessible and innovative treatments for rare conditions.

Fostering Holistic Care

Recognising that rare conditions impact more than just physical health, we launched the EURORDIS Mental Health & Wellbeing Partnership Network. This initiative brings together patients, healthcare professionals, and researchers to address the often-overlooked psychological aspects of living with a rare condition.

Global Awareness and Solidarity

The success of Rare Disease Day 2024, with events in over 100 countries, demonstrates the growing global awareness of rare conditions. The Global Chain of Lights and social media challenges united our community, showing that while rare conditions may be individually uncommon, collectively, we are a powerful force for change.

Looking to the Future

As we move forward, we remain committed to our vision of a world where no one living with a rare condition is left behind. While geopolitical tensions present challenges, they also highlight the rare disease community's strength and adaptability. EURORDIS' work in 2024 proved that collaborative efforts in healthcare and research can serve as a bridge between nations, fostering understanding and cooperation. The challenges ahead are significant, but so is our determination. With the continued support of our members, partners, and the broader rare disease community, we are confident that we will continue to make meaningful progress.

We extend our heartfelt gratitude to everyone who has contributed to our achievements this year. Your dedication, resilience, and passion are the driving forces behind our success.

Together, we are not just hoping for a better future for those impacted by rare conditions – we are actively creating it.

EURORDIS IN BRIEF

AS OF DECEMBER 2024

1997
Year of Establishment

1095
Member Patient Organisations

75
Countries

27
EU Countries

56
National Alliances of RD Patients Organisations

82
European Federations of Specific Rare Diseases

50
Staff, Offices in Paris, Brussels, Barcelona

7 M€
Budget

OVER 60
Volunteers

OVER 2500
Patient Groups Outreach

PHOTO: “RARE IS PROUD”
PRIMARY HYPEROXALURIA - HUNGARY

STRATEGY & IMPACT 2021-2030

In 2020, EURORDIS commissioned an external review for the purpose of developing its strategy for 2021-2030. The strategic review collected input from EURORDIS members and stakeholders, incorporating the work done within the Rare 2030 foresight project that developed recommendations for 2030 and beyond.

The resulting findings and proposed EURORDIS Strategy 2021-2030 were presented and discussed at the General Assembly 2021 and given final validation by the EURORDIS Board of Directors.The Board of Directors continued the strategic review throughout 2022 and adopted the following revised strategy in November.

VISION

EURORDIS' vision is a world where all people living with a rare disease can have longer and better lives and can achieve their full potential, within a society that values their well-being and leaves no one behind.

To achieve their full potential, people living with a rare disease need to be:

- Recognised as equal citizens with their rights fully respected;
- Diagnosed timely and accurately;
- Supported with state-of-the-art medical and social care, or cured;
- Included in society in all aspects of life and enabled to live independently.

MISSION

EURORDIS works across borders and diseases to improve the lives of all people living with rare diseases.

PHOTO: OPEN ACADEMY 2024

OUR STRATEGY

RESEARCH POLICY AND ACTIVITIES

DEVELOPMENT AND ACCESS TO DIAGNOSTICS AND THERAPIES

DATA AND DIGITAL HEALTH

HEALTHCARE POLICY AND SERVICES

SOCIAL POLICY AND SERVICES

PEOPLE FIRST

Based on evidence and experience of unmet needs and preferences.

PARTNER

To establish and facilitate networks with strategic partners and key stakeholders.

EMPOWER

To empower organisations and advocates repre-senting people living with rare diseases across all rare diseases and all European countries.

ADVOCATE

To advocate for policies and innovative solutions driven by the needs of people living with rare diseases.

STRATEGIC OBJECTIVES

A NEW EUROPEAN POLICY FRAMEWORK TO ACHIEVE MEASURABLE GOALS PROLONGING AND IMPROVING THE LIVES OF PEOPLE LIVING WITH RARE DISEASES BY 2030

By 2030, EURORDIS will have promoted and facilitated the implementation of a **new long-term European policy framework** based on the Foresight Study "Rare 2030", guaranteeing that rare diseases are a **sustainable public health priority** and integrating **European policies and national strategies** across all countries in Europe, to **achieve measurable goals improving the health & well-being** of people living with rare diseases and their **inclusion in society, as well as reducing inequalities across Europe.**

By 2030, within the UN Sustainable Development Goals Agenda 2030, the One Sustainable Health Approach and the future European Health Union, we will have designed a **European model of care, transformative and useful beyond rare diseases.** This innovative model will be driven by the needs of people living with a rare disease and a collective responsibility to prioritise solidarity and equity.

DELIVERING ON 6 PRIORITY AREAS

By 2030, EURORDIS will have made contributions to the goals of (Based on the Foresight Study Rare 2030):

- Earlier, faster and more accurate diagnosis – the goal of diagnosis within 6 months;
- High-quality national and European healthcare pathways, including cross-border healthcare – a goal of improving survival by 3 years on average over 10 years and reducing the mortality of children under 5 years of age by one third;
- Integrated medical and social care with a holistic life-long approach and inclusion in society – a goal of reducing the social, psychological and economic burden by one third;
- Research and knowledge development that is innovative and led by the needs of people living with a rare disease;
- Optimised data and health digital technologies for the benefit of people living with a rare disease and society at large;
- Development and availability, accessibility, and affordability of treatments, particularly transformative or curative therapies – a goal of 1000 new therapies within 10 years.

INCLUSIVE OF ALL RARE DISEASES, ALL REGIONS, "LEAVING NO ONE BEHIND"

By 2030, EURORDIS has consolidated its scope to "leave no one behind" in the rare disease community by covering:

- All therapeutic areas, including genetic or non-genetic rare diseases, and rare cancers, with progress to be made with regard to rare infections and rare health hazards;
- All countries in geographical Europe prioritising Eastern and Southern Members of the EU, European Economic Area & EU Accessing Countries;
- All rare disease prevalence and incidence levels, particularly the ones affecting fewer than 1 in 1 000 000.

ORGANISATIONAL MODEL TO FULFIL OUR STRATEGY AND REACH OUR STRATEGIC OBJECTIVES

EURORDIS is a Network Leverager within an ecosystem of networks of member organisations, advocates, partners and stakeholders. Through using our organisation's developed position as a Network Leverager, EURORDIS will achieve its strategic goals.

By 2030, EURORDIS will have catalysed and led impactful motivated networks of member organisations, and of advocates across disease areas, national borders, and age groups. EURORDIS will enable the relay to policymakers of the needs of people living with a rare disease and support the integration of European with national actions in key policy areas.

By the year 2030, EURORDIS will have established itself as a prominent driver of change, leveraging its unique role to initiate and cultivate networks of partners and stakeholders to facilitate the exchange of experiences, co-production of knowledge, and creation of synergies to effectively achieve our strategic objectives.

PHOTO: "NEVER GIVE UP"
PEROXISOMAL BIOGENESIS DISORDER - USA

PHOTO: ARIANA IS LOOKING FOR DOLPHINS IN THE OCEAN" - RUSSIA

PHOTO: 37TH ERTC WORKSHOP, BRUSSELS

HIGHLIGHTS

Key insights from EURORDIS activities and achievements in 2024 include:

Organisational Growth and Engagement

- EURORDIS reached **1,095 members**, adding **63 new members in 2024.** The organisation actively engaged its members through Member News (a bimonthly newsletter in six languages), webinars, Rare Barometer surveys, direct mailings, and the EURORDIS General Assembly;

- Considering the members of its member organisations that are not directly affiliated with EURORDIS, the organisation expanded its outreach to over **2,950 European patient organisations;**

- EURORDIS worked with the **Council of National Alliances (CNA)**, which includes members from **25 EU countries and nine non-EU countries**, supporting National Alliances' advocacy plans and providing tools and information to enhance Member State engagement with relevant EU initiatives and legislation;

- EURORDIS continued to coordinate the **European Network of Disease-Specific European Federations** and the **Council of European Federations (CEF)**, focusing on key strategic issues. This included improving access to **disease-specific data** through the **Rare Barometer surveys** and strengthening **evidence-based advocacy across Europe;**

- EURORDIS supported members of the **Rare Cancer Advocates Network (RCAN)**, which focused on raising awareness for **rare cancer patients**, advancing drug development and access, and ensuring the integration of **rare adult and paediatric cancers** into **National Cancer Plans (NCPs).**

Advocacy and Policy Achievements

- Continued efforts to build support for a European **Action Plan for Rare Diseases**, the main recommendation of the Rare 2030 foresight study. This included advocating towards the European Commission, the European Parliament, and participating in an EESC-organised conference, **For an EU Commitment to Tackling Rare Diseases**, hosted under the Hungarian EU Presidency;

- Actively promoted rare diseases as an **international public health priority** and collaborated with Rare Diseases International (RDI) at the policy level, including participation in RDI's WHO Essential Medicines List Working Group, which aims to improve global treatment access;

- Expanded collaboration with WHO Europe through active involvement in the **Novel Medicines Platform;**

- Organised **Rare Disease Week (RDW) 2024** in Brussels, connecting **19 rare disease advocates from 15 countries** with **27 MEPs, assistants, and EU policymakers**, including representatives from the European Commission and EESC. The event provided advocacy and communication training, policy meetings, and networking opportunities.

Rare Barometer Surveys

- Conducted **large-scale Rare Barometer surveys** on newborn screening (NBS), diagnosis, and disability-related topics;

- As part of the **Screen4Care** project, results from the Rare Barometer survey Voices on Newborn Screening were published in May 2024 (6,179 responses from over 50 countries, covering 1,300+ rare diseases). The results confirmed **strong community support for NBS**, emphasising its role in **reducing diagnostic delays** and **helping parents make informed choices**, regardless of treatment availability. This reinforced EURORDIS' commitment to a **coordinated European NBS approach**, as outlined in its **11 Key Principles for NBS** position paper;

- The **'Journey of Rare Disease Patients to Diagnosis'** survey, the **largest Rare Barometer survey to date**, gathered **13,000 responses globally, including 10,500 from Europe.** The findings highlighted **severe diagnostic delays**, with an **average diagnosis time of nearly five years in Europe.** These results support **national policy initiatives** and **international advocacy efforts** to increase access to **faster and more accurate diagnoses;**

- Launched on 10 July 2024, the **'Rare Barometer Survey on the Impact of Living with a Rare Disease'** focuses on identifying **barriers and enablers to independent living and social participation.** With **10,478 respondents worldwide from 92 countries** (including **9,591 from 43 European countries**), the results are set for release in 2025.

Healthcare and Data Initiatives

- Participated in the newly launched **JARDIN Joint Action** to integrate **European Reference Networks (ERNs)** into national health systems. The initiative addresses key areas such as **care pathways, data interoperability, quality assurance, referral systems**, and **national rare disease plans;**

- **Solve-RD** concluded in 2024. The project collected **21,422 datasets**, conducted **systematic re-analysis and expert reviews on 10,000 individuals**, and achieved a **12.6% diagnostic yield** for previously undiagnosed rare disease patients. Throughout the project, EURORDIS led the Community Engagement Taskforce (CETF), which developed an infographic detailing the patient journey to diagnosis, daily challenges, and available resources;

- Actively contributed to the debate on the **European Health Data Space (EHDS)** and the proposal for a **Regulation on future EU health data provisions.** EURORDIS advocated for the needs and expectations of the **rare disease**

community, ensuring **inclusivity and equity** in Europe's health data landscape and contributing to a stronger, **patient-centred health data ecosystem.**

Research Initiatives

- The **European Joint Programme on Rare Diseases (EJP RD)** concluded in 2024. It brought together **over 130 institutions from 35 countries** to create a **sustainable ecosystem** linking **research, care,** and **medical innovation.** EURORDIS played a key role in leading **patient training** through **Open Academy courses** and coordinating the **Patient Engagement in Research Working Group**, which led to the publication of the **Patient Partnerships Guide** and contributed to the development of **MOOCs;**

- **ERDERA (European Rare Diseases Research Alliance and Partnership)** was launched in September 2024 and will run until August 2031 under Horizon Europe. This major collaboration involves EURORDIS and key European rare disease research stakeholders, spanning **36 countries** and **171 organisations** as full partners. EURORDIS co-leads the **Education and Training Work Package**, leads the **Patient and Public Involvement and Engagement (PPIE) Group**, and contributes to research on the **socioeconomic impact of rare diseases** and the identification of disease indications requiring **Advanced Therapy Medicinal Products (ATMPs)**;

- **RD Moonshot Translational Research Needs Recommendations** were finalised and disseminated online, outlining the **value of public-private partnerships** in rare disease research.

Patient Engagement and Education

- **Massive Open Online Courses (MOOCs)** co-developed by EURORDIS under EJP RD remain available after the end of the project;

- Organised **Barcelona Open Academy courses** (Medicines Research & Development course and Scientific Innovation & Translational Research course) training **70 participants from 23 countries**, with a v**ery high satisfaction rate;**

- Expanded the Open Academy **e-learning platform** to 4,**809 registered users** from **172 countries;**

- Launched **EUCAPA** to prepare patients and patient representatives **for meaningful** involvement in health technology assessment (HTA) **ahead of the EU HTA Regulation (EU) 2021/2282 (HTAR), which takes effect in January 2025.**

Treatment Development and Access

- Worked on the **revision of the General Pharmaceutical Legislation**, with initial efforts focused on the European Parliament, which successfully adopted its position on **10 April 2024**, just before the end of its **five-year mandate.** EURORDIS played a key role in advocating for amendments that were ultimately included in the final position, strengthening the **European Commission's** original proposals. A **targeted outreach programme** was then launched to strengthen contact with key **health attachés** in Brussels, ensuring rare diseases remain a priority in Council-level discussions;

- Supported **patient experts** in participating in **EMA** scientific advice and regulatory discussions. EURORDIS identified, mentored, and supported **21 patient experts** (providing input on 84% of dossiers requiring patient involvement)

to participate in **scientific advice and protocol assistance activities.** Through its extensive involvement in **EMA committees and working parties**, EURORDIS continues to ensure the voice of people living with rare diseases is integrated into European regulatory decision-making;

- Participated in projects such as **REMEDi4ALL** (focused on the development and implementation of repurposed medicines) and **JOIN4ATMP** (designed to accelerate and de-risk the development of **Advanced Therapy Medicinal Products (ATMPs)**) to advance drug development and improve treatment access;

- Advocated for **enhanced access to authorised therapies** for rare diseases across the EU. Notably, the European Commission's EU4Health programme for 2024 introduced a call for Member States to support structured cooperation in pricing and reimbursement policies.

Holistic Care

- As a member of the **European Disability Forum (EDF) and the EU Disability Platform**, EURORDIS advocated for **the implementation of the European Strategy for the Rights of Persons with Disabilities 2021-2030**, highlighting the **barriers faced by people with rare diseases** and advocating for their access to the **European Disability Card and independent living support**;

- Launched the **EURORDIS Mental Health & Wellbeing Partnership Network**, bringing together **137 members from 28 countries** (19 EU Member States and 9 non-EU countries). The Network has strengthened ties within the rare disease community, engaging **more than 80 experts with lived experience** and their representatives, alongside **60 medical professionals, psychologists, and researchers from 19 European Reference Networks (ERNs) and other international rare disease centres.**

Awareness and Global Outreach

- Celebrated outstanding contributions to the rare disease community, honouring patient advocates, organisations, policymakers, scientists, companies, and media for their impact at the **EURORDIS Black Pearl Awards 13th Edition;**

- Organised the **17th edition of Rare Disease Day**, with **over 1,000 events in more than 100 countries.** Events ranged from **political conferences and medical symposia to sports activities and community gatherings;**

- The **#RareDiseaseDay challenge** encouraged global participation through themed daily social media posts. Despite energy constraints, **monuments, homes, and offices worldwide were illuminated** as part of the **Global Chain of Lights**, showing solidarity with the rare disease community;

- Organised **ECRD 2024** in May in Brussels as a fully hybrid conference. ECRD is the largest **patient-led, rare disease policy-shaping event in Europe.** With **over 700 participants**, the event culminated in the publication of a **co-created open letter to EU Institutions and country leaders.**

STRATEGIC OBJECTIVE 01:

A NEW EUROPEAN POLICY FRAMEWORK TO ACHIEVE MEASURABLE GOALS PROLONGING AND IMPROVING THE LIVES OF PEOPLE LIVING WITH RARE DISEASES BY 2030

Throughout 2024, EURORDIS has **supported this strategic objective through advocacy, empowerment and partnership activities** and continued to promote rare diseases as a policy priority at national, European, and international levels.

ADVOCATE | EMPOWER | PARTNER

PHOTO: OPEN ACADEMY 2024

ADVOCATE

ADVOCATING FOR AN UPDATED POLICY FRAMEWORK FOR RARE DISEASES AND RARE CANCERS FOR 2021-2030

The campaign for a European Action Plan for Rare Diseases

Throughout 2024, efforts continued to build support for a **European Action Plan for Rare Diseases**, the main recommendation of the Rare 2030 foresight study. Key activities included:

- Contributing to and speaking at the EESC-organised conference, **"For an EU Commitment to Tackling Rare Diseases",** hosted under the Hungarian EU Presidency. The event reinforced the need for harmonised European policies and strengthened healthcare systems;
- **Supporting Members of the European Parliament** in sending a letter to European Commission leaders **urging the adoption of an EU Action Plan for Rare Diseases.**

Involving the European Network of National Alliances in advocacy activities

EURORDIS supported National Alliances through in-person CNA meetings, monthly «hot topics» calls, and shared advocacy plans. Throughout the year, EURORDIS shared advocacy plans and supplied tools and information to National Alliances in order to enhance Member State engagement with relevant EU initiatives and legislation. The EURORDIS-CNA Common Goals and Mutual Commitments were updated and approved in May 2024.

PROMOTING RARE DISEASES AS AN INTERNATIONAL PUBLIC HEALTH PRIORITY WITH RARE DISEASES INTERNATIONAL

EURORDIS has been working on **the promotion of rare diseases as an international public health priority for almost a decade.** The aim has been to raise awareness of rare diseases among relevant international institutions; to equip patient groups with advocacy tools to engage their national authorities and drive local patient empowerment; and to foster greater international cooperation in the field of rare diseases.

RDI (Rare Diseases International):

The **major key RDI Policy Achievements that have been co-led by EURORDIS** so far are:

- **Inclusion of 'persons living with rare diseases' in the 2nd UN Political Declaration on Universal Health Coverage** (UHC) (Sept. 2023);
- **Strengthened UN General Assembly resolution on rare diseases**, reinforcing universal health coverage and diagnostics (Nov. 2023);
- **RDI's Non-State Actor (NSA) status application to WHO**, expected to benefit EURORDIS within the WHO-Europe Region.

EURORDIS remained a key RDI member in 2024:

- Yann Le Cam participated in six Council meetings and chaired the RDI Policy Committee, as a member of the RDI council;
- Virginie Hivert contributed to RDI's WHO Essential Medicines List Working Group aimed at improving global treatment access;
- Continued collaboration with RDI and the UN NGO Committee for Rare Diseases to integrate rare diseases into international policy frameworks.

WHO Regional Committee for Europe

EURORDIS expanded its collaboration with WHO Europe in 2024 through **active involvement in the Novel Medicines Platform**, a three-year initiative addressing access to innovative treatments. EURORDIS representatives participated in two of three Working Groups. EURORDIS attended the launch of the NMP in Copenhagen (July 2024) and this initiative is set for 3 years. In addition, EURORDIS took on the **co-leadership on the European Access Dialogue Platform proposal**, aimed at increasing treatment access across the WHO Europe Region (2025-2027). These sustained efforts ensure rare diseases remain a priority within EU and international policy frameworks, improving access to healthcare and treatment for people living with rare diseases worldwide.

PHOTO: OPEN ACADEMY 2024

PHOTO: EURORDIS MEMBERSHIP MEETING 2023

EMPOWER

STRENGTHENING OUR NETWORKS: COMMUNITY BUILDING, CAPACITY DEVELOPMENT, INFORMATION SHARING, AND SUPPORT

Membership

EURORDIS continued its steady growth in 2024, welcoming **63 new members and reaching a total of 1,095.** The organisation actively engaged members through Member News (a bimonthly newsletter in six languages), webinars, Rare Barometer surveys, direct mailings, and the EURORDIS General Assembly. Outreach extended to over 2,950 European patient organisations, enhancing the contact database.

European Network of Rare Disease National Alliances and the Council of National Alliances

The National Alliances consolidate numerous rare disease organisations within a specific country. The Council of National Rare Disease Alliances (CNA), established and coordinated by EURORDIS, facilitates collaboration among national representatives of rare disease patients on common European actions.

Significant achievements of the CNA include:

- The creation and successful implementation of Rare Disease Day;
- National Alliances actively promoting and developing national plans for rare diseases;
- The adoption of the «Common Goals and Mutual Commitments between EURORDIS & National Alliances in Europe»;

- Leading a European campaign for a new policy framework;
- Advocacy for integrating European Reference Networks (ERNs) at the national level;
- Development of EURORDIS' position on newborn screening.

Currently, the CNA includes members from 25 EU countries and 9 non-EU countries (Bosnia, Georgia, North Macedonia, Montenegro, Norway, Ukraine, Russia, Serbia, Switzerland). In 2024, the CNA conducted two workshops: the first in May, a one-day meeting and the second in December, spanning two days with the first day dedicated to the CNA and the second involving both CNA and CEF representatives. CNA members convene online monthly for «CNA Hot Topics» discussions and receive the «CNA Update» monthly newsletter.

European Network of Disease-Specific European Federations and the Council of European Federations

European Federations, coordinated by EURORDIS, unite national patient organisations specific to rare diseases at the European level. The CEF facilitates collaboration on shared European initiatives.

In 2024, EURORDIS continued its coordination of the European Network of Disease-Specific European Federations and the CEF, focusing on key strategic issues. This included **improving access to disease-specific data through the Rare Barometer surveys**, aiding evidence-based advocacy across Europe. Furthermore, **EURORDIS provided financial support for network meetings** of 16 emerging or less established rare disease European Federations.

Representatives of European Rare Disease Federations gathered in Paris in October 2024 jointly with the CNA to discuss among other topics, the unmet need of mental health for people living with rare diseases and the EU Joint Clinical Assessment.

European Network of Rare Disease Helplines

Established in September 2006 and coordinated by EURORDIS, the **ENRDHL** aims to enhance service to callers by sharing resources, best practices, and knowledge among rare disease helplines. The network strives to raise awareness, increase efficiency, and improve practice standards across its membership.

A key activity of the ENRDHL is the **Caller Profile Analysis**, an annual review conducted each October, where enquiries from all helplines are consolidated to analyse 13 common items, enhancing understanding of caller needs and trends.

Brussels Rare Disease Week 2024

Rare Disease Week (RDW) 2024, organised by EURORDIS-Rare Diseases Europe in Brussels, connected rare disease advocates with MEPs and EU policymakers through training, policy meetings, and networking events. The programme provided advocacy and communication training while fostering relationships to raise awareness and deliver key policy messages.

With the 2024 European Parliament elections marking a new term, **RDW played a crucial role in renewing EURORDIS' network of MEP advocates and equipping patient advocates with the skills and confidence to engage in advocacy at all levels.**

Participants gained insights into EU structures, legislative processes, and advocacy strategies through e-learning, skill sessions, and interactive engagement with policymakers. A dedicated toolkit supported their efforts with policy priorities and key messages.

Held from 18 to 20 November 2024 in Brussels, RDW welcomed 19 participants from 15 countries. They met with 27 MEPs and assistants, engaged with EU Commission and EESC representatives, and attended a networking lunch at the European Parliament.

Black Pearl Awards (BPA) 2024

The EURORDIS **Black Pearl Awards** celebrate outstanding contributions to the rare disease community, honouring patient advocates, organisations, policymakers, scientists, companies, and media for their impact. Held annually in February alongside Rare Disease Day, the **13th edition** took place on **20 February 2024** at the **DoubleTree by Hilton Brussels City**, with an online broadcast. The event gathered **330 attendees from 42 countries** across all stakeholder groups.

PHOTO: RARE DISEASE WEEK 2024 PARTICIPANTS

PHOTO: SILVIA AGUILERA, EURORDIS VOLUNTEER AWARDEE 2024

2024 Award Winners:

- **Company Award for Health Technology** (with MedTech Europe): Insension Initiative / Poznan Supercomputing Networking Centre;
- **Company Award for Innovation:** Sanofi;
- **Company Award for Patient Engagement:** Pierre Fabre & EspeRare Foundation;
- **European Rare Disease Leadership Award:** Dr Hans Kluge;
- **Policy Maker Award:** Alain Coheur;
- **Young Patient Advocate Award:** Adéla Odrihocká;
- **Scientific Award** (with Orphanet): Prof. Jose-Alain Sahel;
- **EURORDIS Members Award:** DEBRA International;
- **EURORDIS Volunteer Award:** Silvia Aguilera;
- **Holistic Care Award:** RareResourceNet;
- **Media Award:** Never Stop Moving (Neuromuscular Diseases);
- **Social Media Award Finalist:** Alba Parejo.

Each award highlights excellence in leadership and advocacy, inspiring continued progress in rare disease research, care, and policy.

European Conference on Rare Diseases and Orphan Drugs (ECRD) May 2024

The **12th edition of the European Conference on Rare Diseases and Orphan Products (ECRD)** was fully hybrid for the first time. This new format ensured the conference remained accessible for all, whilst also providing the opportunity for stakeholders to come together in Brussels ahead of the 2024 European Parliamentary elections. The event had a combined audience of **over 700 participants from across 49 countries.**

Throughout the two-day programme, participants were able to take a deep-dive into discussions on how EU institutions and country leaders can keep **rare diseases as a key focus within healthcare policy.** A number of pre-conference Thought Leader Sessions, alongside a library of resources including podcasts, videos and research

papers, were organised in advance of the conference to bolster the learning experience for registered and prospective attendees.

This year's conference culminated in the **co-signing of the EURORDIS Open Letter**; an official summary of the **policy asks and key-takeaways from ECRD 2024**, to be presented to the European Commission. The **Executive Summary** for the event was **published online.**

PHOTO: "THE WORLD HAS THE COLOURS THAT WE PAINT"
AUTOIMMUNE ENCEPHALITIS - BRAZIL

STRATEGIC OBJECTIVE 02:

DELIVERING ON PRIORITY AREAS

EURORDIS has undertaken a range of activities to achieve this Strategic Objective, in alignment with its overarching strategy to advocate for, empower, and partner with people living with rare diseases. These activities fall within key priority areas: Healthcare Policy and Services, Research Policy and Activities, Data and Digital Health, Development and Access to Diagnostics and Therapies, and Social Policy and Services.

HEALTHCARE POLICY AND SERVICES

RESEARCH POLICY AND ACTIVITIES

DATA AND DIGITAL HEALTH

DEVELOPMENT AND ACCESS TO DIAGNOSTICS AND THERAPIES

HOLISTIC CARE

PHOTO: OPEN ACADEMY 2024

HEALTHCARE POLICY AND SERVICES

01 05

"EARLIER, FASTER AND MORE ACCURATE DIAGNOSIS: THE GOAL OF DIAGNOSIS WITHIN SIX MONTHS"

HIGH-QUALITY NATIONAL AND EUROPEAN HEALTHCARE PATHWAYS, INCLUDING CROSS-BORDER HEALTHCARE: THE GOAL OF IMPROVING SURVIVAL BY 3 YEARS ON AVERAGE OVER 10 YEARS AND REDUCING THE MORTALITY OF CHILDREN UNDER 5 YEARS OF AGE BY ONE THIRD"

ADVOCATE

ADVOCATING TO IMPROVE ACCESS TO QUALITY RARE DISEASE DIAGNOSIS

Newborn screening

The **Newborn Screening Working Group (NBS WG)**, composed of representatives from **patient organisations, screening societies, and federations**, works to advance **harmonised newborn screening (NBS) programmes** across Europe. Its goal is to **maximise benefits and improve health outcomes** for babies born with rare diseases.

In 2024, EURORDIS reinforced its commitment to a **coordinated European NBS approach**, as outlined in its **11 Key Principles for NBS** position paper. Key actions included:

- **Advocating for an EU-level expert working group** to support **harmonised NBS strategies** across Member States;
- **Leading the EURORDIS NBS WG**, driving research, policy activities, and promoting the **11 Key Principles;**

- **Publishing findings** from the Rare Barometer survey Voices on Newborn Screening in **May 2024**, as part of the **Screen4Care** project. The results confirmed **strong public support for NBS**, emphasising its role in **reducing diagnostic delays** and **helping parents make informed choices**, regardless of treatment availability.

EURORDIS Rare Barometer Programme: Patient experience surveys enabling evidence-based contributions to policymaking and research

The **Rare Barometer programme** collects **qualitative and quantitative data** on the experiences, needs, and expectations of people living with a rare disease. With **over 20,000 participants**, its surveys inform **EURORDIS' advocacy efforts** and are translated into **27 languages** to ensure broad impact.

Rare Barometer Survey: The journey of rare disease patients to diagnosis

A **peer-reviewed article** on the diagnosis survey was published in the **European Journal of Human Genetics (May 2024)**, with key findings presented at the **12th European Conference on Rare Diseases (ECRD) to 300 attendees.** As the **largest Rare Barometer survey to date** (13,000 respondents globally, 10,500 in Europe), it highlighted **severe diagnostic delays**, with an **average diagnosis time of nearly five years** in Europe. These findings support **national policy initiatives and international advocacy efforts.**

PHOTO: SCREEN4CARE PROJECT'S NEWBORN SCREENING (NBS) FORUM IN BARCELONA. OCTOBER 2024

Rare Barometer Survey: Newborn Screening

Conducted with experts, policymakers, and geneticists, this survey gathered **6,179 responses from over 50 countries**, covering **1,300+ rare diseases**. Published in **April 2024**, results were shared through:

- An **online webinar** (159 registrants, 84 attendees, 101 YouTube views);
- A **comprehensive report** (38 pages, English) and a **European factsheet** (14 languages);
- **63 national factsheets and 144 online dashboards** (available in 25 languages) shared with **national alliances, European federations, and ERNs;**
- A **poster presentation** at the **New York International Conference on Newborn Screening (ICoNS) (July 2024).**

Findings confirmed **strong community support for newborn screening at birth.**

Rare Barometer Survey: Social participation and independent living

Launched on **10 July 2024**, this survey addressed **disability-related topics**, with **10,478 respondents worldwide (9,591 in Europe).** Key outreach efforts included:

- A **webinar** (200 registrants, 109 attendees, 117 YouTube views);
- A **communications toolkit in 25 languages;**
- **133 online dashboards**, distributed to **National Alliances, European Federations, ERNs, and EURORDIS members.**

The survey closed in **September 2024**, with **results set for publication in 2025.** However, **preliminary findings** were discussed with **National Alliances and European Federations** at the **CNA-CEF meeting in Paris (Nov. 2024).**

EMPOWER

SUPPORTING PATIENT PARTNERSHIP WITHIN THE EUROPEAN REFERENCE NETWORKS (ERNS)

Since **2016**, EURORDIS has been instrumental in **patient involvement** within the **ERNs**, establishing **24 European Patient Advocacy Groups (ePAGs).** These groups, composed of **over 300 patient representatives**, facilitate collaboration between **patient organisations, clinicians, and researchers, ensuring patient perspectives are integrated into ERN decision-making.**

In **2024**, EURORDIS continued to **strengthen patient partnerships** within ERNs by enhancing **patient-clinician collaboration, developing new resources, and supporting patient representative involvement.**

Strengthening patient-clinician partnership in the ERNs

EURORDIS fostered collaboration through **peer-learning, resource development, and knowledge exchange.** In **2024**, six **webinars** were organised, sharing **best practices** on **patient partnership in education, evidence evaluation, and hospital service improvements.**

Additionally, EURORDIS developed three key resources:

- **Project Planning Toolkit for Patient Partnership;**
- **Workshop Toolkit: Patient Involvement in Clinical Practice Guidelines (CPGs);**
- **Patient Journey Templates for ERNs.**

To centralise tools and resources, **a new Patient Partnership Hub** was launched, building on the work of the **ePAG Knowledge Management Working Group.** A **survey** assessed its usage, with **174 responses from 19 ERNs.** Findings showed that **33% had used at least one tool**, with **patient representatives as the primary users.** Suggestions for improvement included **short explanatory videos and real-world examples** of tool implementation.

Enabling and supporting Patient Representative Involvement in ERNs

EURORDIS provided **ongoing support and training** for patient representatives through **ePAG transversal working groups**, ensuring they had the **knowledge and skills** to engage effectively with clinicians. In **2024**, EURORDIS managed **four transversal ePAG Working Groups**, conducting **13 meetings:**

- **ePAG Steering Committee (5 meetings):** Drafted a **Board of Member States (BoMS) statement** on **formalising patient representation in ERNs;**
- **Patient Partnership Working Group (3 meetings):** Developed and piloted **new patient engagement tools;**
- **Knowledge Management Working Group (4 meetings):** Launched the **Patient Partnership Hub;**
- **ePAG AMEQUIS Task Force (1 meeting):** Reviewed the **five-year ERN evaluation report.**

Beyond working groups, EURORDIS participated in **over 70 individual ePAG meetings**, attended **six annual ERN meetings**, and organised **two induction sessions** for new patient representatives in **March and October**, with **52 participants** and a **100% satisfaction rate.**

Raising Awareness and working with NAs to connecting Patient Organisations to ERNs

EURORDIS has contributed to disseminating and raise awareness on ERNs through the ePAG Quarterly Newsletters and the Quarterly episodes of the “ERNs on Air” podcast.

PHOTO: COUNCIL OF NATIONAL ALLIANCES MEETING. PARIS. 2024

PARTNER

H-CARE Project

Launched in **2019**, the **H-CARE project** was initiated by **ERNs ERKNet, eUROGEN, GENTURIS, and LUNG**, with support from the **EURORDIS Rare Barometer Programme.** It aims to develop a **European feedback mechanism** using **Patient Reported Experience Measures (PREMs)** to evaluate the **healthcare experiences of people living with rare diseases.**

In **2024**, significant progress was made:

- A **scoping literature review**, conducted with **Genetic Alliance UK and Heidelberg University, was submitted to the Orphanet Journal of Rare Diseases (December 2024).** Preliminary results were presented at the **12th European Conference on Rare Diseases;**
- The project received **ethics approval from University College Dublin (UCD)**, allowing for **expert consultations and patient focus groups;**
- Discussions at the **CNA meeting** led to the identification of **new patient organisation partners** and engagement with **PREM development experts;**
- EURORDIS joined the **Rare Disease Research Catalyst Consortium (RDCat)** to enhance **patient involvement in research.** Going forward, **RDCat will coordinate the project**, with **EURORDIS remaining a key collaborator**, working closely on **PREM development.**

Joint Action on the integration of European Reference Networks into national health systems (JARDIN, January 2024-2027, DG Sante, Subcontractor).

Launched in **January 2024**, the **JARDIN Joint Action** (funded by **DG SANTE**) aims to **integrate European Reference Networks (ERNs) into national health systems**, addressing key areas such as **care pathways, data interoperability, quality assurance, referral systems, and national rare disease plans.** The project kicked off in **Brussels (6-8 March 2024)**, with **EURORDIS contributing to three work packages** focused on:

- **Refining care pathway methodologies** and developing **models for 10 diseases;**
- **Developing case manager guidelines;**
- **Improving data sharing** between **national health systems and ERN registries;**
- **Creating emergency response recommendations** for rare disease patients;
- **Designing models for patient involvement** in clinical networks;
- **Developing strategies for undiagnosed pathways** and linking them to ERNs;
- **Establishing an ERN-overarching expert panel** for complex undiagnosed cases;
- **Supporting the creation of patient organisations** for undiagnosed patients in Member States.

Several **National Alliances** – including **UNIAMO (Italy), RD Ireland, ACHSE (Germany), Rare Diseases Greece, and Pro-Rare Austria** – are involved in the project and receive funding for specific tasks.

Progress in 2024:

- **Model care pathways:**
  - **15 ERNs** committed to developing care pathways, with **9 already selecting a topic area and clinical lead.**
  - A **model process and patient involvement toolkit** were developed to support pathway creation.

- **National Reference Networks & Patient Partnership:**
  - EURORDIS co-developed **recommendations and a toolkit** for patient partnership in **National Reference Networks.**
  - The recommendations will be refined based on a **survey mapping governance structures** of existing networks.

- **Support for Undiagnosed Patients:**
  - EURORDIS contributed to a **survey on structures and models supporting undiagnosed patients** at national and international levels.
  - An **Undiagnosed Multistakeholder Task Force** was established, bringing together **26 members from 12 countries** to develop **recommendations for undiagnosed patient support.**

PHOTO: THE LAUNCH OF THE JARDIN JOINT ACTION

Collaborative project on diagnostic characterisation of rare diseases (Solve-RD, January 2018-March 2024, Horizon 2020, Partner)

Solve-RD, a Horizon 2020-funded project (January 2018–March 2024), aimed to **identify the molecular causes of undiagnosed rare diseases.** It aligned with the objectives of the **International Rare Diseases Research Consortium (IRDiRC)** to develop **diagnostic tests for most rare diseases.** By its conclusion in **March 2024**, the project had collected **21,422 datasets**, conducted **systematic re-analysis and expert reviews on 10,000 individuals**, and achieved a **12.6% diagnostic yield.**

EURORDIS played a key role in **stakeholder engagement and patient involvement**, contributing through:

- **Steering Committee participation**, ensuring multi-stakeholder engagement;
- **Leading the Community Engagement Task Force (CETF)**, facilitating collaboration between **patients, scientists, and clinicians;**
- **Developing an infographic on the diagnostic journey**, translated into **29 languages;**
- **Contributing to a policy paper**, advocating for **improved rare disease diagnostics across Europe.**

Solve-RD **officially concluded on 31 March 2024**, with its findings laying the groundwork for **future rare disease research initiatives and enhanced diagnostic strategies.**

RESEARCH POLICY AND ACTIVITIES

02 05

"RESEARCH AND KNOWLEDGE DEVELOPMENT THAT ARE INNOVATIVE AND LED BY THE NEEDS OF PEOPLE LIVING WITH A RARE DISEASE"

ADVOCATE

Rare Disease Moonshot

The **Rare Disease Moonshot** is a coalition of seven organisations – **Critical Path Institute (C-Path), European Infrastructure for Translational Medicine (EATRIS), European Clinical Research Infrastructure Network (ECRIN), European Federation of Pharmaceutical Industries and Associations (EFPIA), European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), EuropaBio, and EURORDIS-Rare Diseases Europe.** It aims to **accelerate scientific discovery and**

drug development for rare and paediatric diseases without existing treatments by **fostering collaboration, reducing research fragmentation, and strengthening public-private partnerships** in research.

Since its launch in **December 2022**, EURORDIS has contributed through **regular bimonthly or monthly calls.** In **May 2024**, EURORDIS organised an **ECRD pre-webinar**, "Collaborating for Change-Transforming Rare Disease Outcomes through Public-Private Partnerships," co-powered by **Together4RD and the Rare Disease Moonshot.** The session presented **best practices, opportunities, challenges, and the role of PPPs in Europe and beyond.** In **2024**, the **RD Moonshot Translational Research Needs Recommendations** were **finalised and disseminated online**, outlining the **value of public-private partnerships** in rare disease research.

To further promote the initiative, several **publications were released**, including an **article in the DIA Global Forum (January 2024)** and another in **Clinical Pharmacology & Therapeutics**, "The Rare Disease Moonshot: Paradigm Shift, Translational Medicine, and Regulatory Science for the World's Rarest Conditions" (August 2024). To mark its **second anniversary**, EURORDIS, represented by **board member Graham Slater**, joined **ERDERA, EFPIA, and MEP Voiculescu in a Euronews debate (November 2024)**, "Innovating for Impact: Shaping the Future of Rare Disease Treatment", discussing **progress, challenges, and the systemic changes needed to ensure lasting impact.**

EMPOWER

EURORDIS Open Academy

The **EURORDIS Open Academy** provides **patient advocates** with the **skills and knowledge** needed for **effective engagement** in rare disease advocacy at both **European and national levels.** It offers **in-depth training through schools, alumni meetups, masterclasses, and open-access e-learning courses** available on the **Open Academy website.**

By the end of **2024**, the **e-learning platform** had **4,809 registered users from 172 countries.** Throughout the year, EURORDIS continued to **engage Open Academy alumni** through **4 Alumni Meetups and 2 Masterclasses**, each focusing on a **specific topic relevant to patient advocates.** These sessions received a **high satisfaction rate** from attendees. Additionally, **11 newsletters** were sent to alumni and platform users, sharing updates on the **Open Academy and other training opportunities.**

The **17th edition of the EURORDIS School on Medicines Research & Development** was held in **Barcelona from 3-7 June 2024**, bringing together **31 trainees and 7 researchers from 25 countries.** The training covered **key topics supporting patient engagement in medicine research and development.**

The **7th edition of the School on Scientific Innovation & Translational Research took place alongside the Medicines Research & Development School**, also in **Barcelona from 3-7 June 2024**, with **29 trainees**

and 3 researchers from 18 countries. The programme focused on **enhancing patient engagement in scientific research**, with two shared **training days between the two schools** to encourage interdisciplinary learning.

PHOTO: EURORDIS OPEN ACADEMY 2024

PARTNER

TRANSLATIONAL RESEARCH

ERDERA (European Rare Diseases Research Alliance and Partnership, September 2024 - August 2031, Horizon Europe Partner)

ERDERA (European Rare Diseases Research Alliance and Partnership) was **launched on 1 September 2024** and will run until **August 2031** under **Horizon Europe**. It represents a major collaboration involving **EURORDIS** and key European rare disease research stakeholders. EURORDIS played a pivotal role in **co-designing ERDERA**, contributing to the **Strategic Research and Innovation Agenda (SRIA)** and the **project proposal submitted in September 2023**.

The project involves **36 countries** and **171 organisations** as full partners, with an overall estimated budget of **€385.5 million**, co-funded by the **European Commission, European Member States, and additional partners**.

EURORDIS is involved in **10 of the 24 work packages** and **co-leads the Education and Training work package**. It also leads the **Patient and Public Involvement and Engagement (PPIE) Group** and contributes to research on **the socioeconomic impact of rare diseases** and **identifying disease indications needing Advanced Therapy Medicinal Products (ATMPs)**. The project is expected to involve **three to four full-time EURORDIS staff**, collaborating with patient organisations, including **AFM-Téléthon, World Duchenne Organisation, Genetic Alliance UK, RDI, and the Thalassemia International Foundation**.

Progress in 2024:

- **SRIA public consultation** was finalised in **October**;
- **ERDERA kick-off meeting** took place in **Paris (28–29 October)**, with EURORDIS **chairing sessions** and aligning objectives among partners;
- **Workshop prepared** to map **patient organisation efforts in rare disease research** and facilitate engagement with **ERDERA's funding mechanisms**;
- **Scoping review and multistakeholder survey** initiated to identify **rare diseases with the greatest need and suitability for ATMPs**;
- **Open Academy onsite courses** for **2025** were organised, along with a new **online Data Pilot School** set to launch in **February 2025**;
- **Support for National Alliances** in establishing **ERDERA National Mirror Groups**, strengthening **patient involvement at the national level**.

PHOTO: ERDERA KICK-OFF MEETING 2024

European Joint Programme on Rare Diseases (EJP RD, January 2019 – August 2024, Horizon 2020, Partner)

The **European Joint Programme on Rare Diseases (EJP RD)**, running from **January 2019 to August 2024** under **Horizon 2020**, brought together **over 130 institutions from 35 countries** to create a **sustainable ecosystem linking research, care, and medical innovation**. The programme focused on **scaling and optimising existing tools**, ensuring they meet **end-user needs** through **real-world implementation tests**.
2024 Highlights

The **Barcelona Open Academy courses** trained **70 participants from 23 countries**, receiving a **very high satisfaction rate** from attendees.

Three **Massive Open Online Courses (MOOCs)** co-developed by EURORDIS under EJP RD remain available:

- **Diagnosing Rare Diseases: from the Clinic to Research and Back** (6,380 learners, 140 countries);
- **From Lab to Clinic: Translational Research for Rare Diseases** (2,203 learners);
- **Health Data Ethics & Regulatory Frameworks in Rare Disease Research** (launched May 2024, 633 learners from 81 countries).

Patient Engagement in Research (PENREP Working Group)

EURORDIS coordinated a **Patient Engagement in Research workshop** in **May 2024 (Bari, Italy)** during the evaluation meeting for **EJP RD 2021-funded Social Sciences & Humanities projects**. A **pre-workshop survey (March-April 2024, 26 respondents)** revealed **discrepancies between patient and researcher perspectives on engagement**, reinforcing the **need for stronger support** in both **biomedical and social science research**.
Final EJP RD General Assembly Meeting
EURORDIS played an active role in **agenda planning, proposing sessions, and inviting speakers**. A **pre-ECRD podcast** was recorded with **Daria Julkowska, Holm Graessner, and Roseline Favresse**, discussing the **transition to ERDERA** and its upcoming launch.

Key Outcomes of the EJP RD Training & Education Pillar

- **1,023 trainees** participated in **Data Management & Quality Trainings**, with representation from **51 countries**, including **20 underrepresented countries**;
- **485 trainees** (including **7% researchers**) attended **patient training programmes** from **50+ countries**;
- **91 ERN fellowships** and **28 ERN workshops** were organised, involving fellows from **26 countries** (including **18 underrepresented countries**) and workshops in **10 countries** (including **3 underrepresented countries**);
- **8,000+ learners** enrolled in **MOOCs**, representing **150+ countries**, with **40% outside Europe**.

European Rare Disease Research Coordination and Support Action (ERICA, March 2021 – February 2025, Horizon 2020, Partner)

The **European Rare Disease Research Coordination and Support Action (ERICA)**, running from **March 2021 to February 2025** under **Horizon 2020**, aims to **enhance the research and innovation capacities of European Reference Networks (ERNs)** by creating an **integrated platform** for

collaboration. The project focuses on **strengthening ERNs through competitive research networks**, **improving data collection**, **enhancing patient involvement**, **optimising clinical trials**, and **highlighting ERNs' innovation potential**. By aligning ERN activities with European research infrastructures, ERICA seeks to **maximise impact and drive innovation** in rare disease research.

In **2024**, EURORDIS **finalised a Patient Partnership Framework for Clinical Trials**, co-developed with **ERN clinicians and ePAG advocates**. The framework was **refined and validated** throughout the year before being **disseminated to project partners**. The **final deliverable is set for submission in February 2025**.

Undiagnosed Diseases Network International

The **Undiagnosed Diseases Network International (UDNI)** brings together **clinicians, researchers, genetic counsellors, and medical professionals worldwide** to address **complex and unresolved medical cases**. A key aspect of UDNI's approach is **patient and patient representative involvement**, ensuring a **patient-centric focus** in all activities. Established in **2014**, UDNI was developed to meet the **global needs of undiagnosed patients**, with **EURORDIS, NORD, and the Wilhelm Foundation** playing a crucial role in creating a **patient engagement membership**, later endorsed by **UDNI's Board**.

In **2024**, EURORDIS continued its engagement with UDNI by:

- **Hosting online meetings** to strengthen **visibility and collaboration**;
- **Organising a video project** to highlight **contributions from patient organisations**;
- **Promoting member activities** for **Undiagnosed Day**;
- **Presenting at the 13th International UDNI Conference**, emphasising the importance of **patient involvement**.

PAEDIATRIC CLINICAL TRIALS

Conect4Children (C4C, May 2018-April 2024, extended until April 2025, IMI 2, Partner)

Conect4Children (c4c) is a **pan-European research network** uniting **35 academic institutions, 10 industry partners, and over 500 affiliated partners** across **20 European countries**. It brings together **pharmaceutical companies, national paediatric networks, multinational subspecialty networks, children's hospitals, and patient advocacy groups** to accelerate the **development of new drugs and therapies for children**.

Since **2019**, EURORDIS has been an **active member of the c4c Multistakeholder Programme Committee**, playing a key role in **organising Multi-Stakeholder Meetings (MSM)** on various **paediatric conditions**, including **inflammatory bowel disease (2020), type 1 diabetes (2022), perinatal asphyxia (2023), paediatric irritability (2024).**

These meetings integrate input from **young patients, parents/carers, and patient advocates**, contributing to **peer-reviewed scientific publications** on MSM outcomes. EURORDIS has also **led workshops for patient organisations on paediatric drug development** and hosted **follow-up webinars for patient experts**. Additionally, it is involved in drafting a **white paper** on c4c's experience with **parents and young patients**.

Beyond advocacy, EURORDIS contributes to **c4c's work on data quality standards** and is part of the **Education and Training Programme** as a member of the **Education Board**.

The **c4c project has been extended until April 2025** and will then transition into **c4c Stichting/Foundation**, where EURORDIS will continue its involvement.

DATA AND DIGITAL HEALTH 03 05

"OPTIMISED DATA AND HEALTH DIGITAL TECHNOLOGIES FOR THE BENEFIT OF PEOPLE LIVING WITH A RARE DISEASE AND SOCIETY AT LARGE"

ADVOCATE

Advocating for a European Health Data Space

EURORDIS actively contributed to the debate on the **European Health Data Space (EHDS)** and the proposal for a **Regulation establishing future EU health data provisions** by advocating for the **needs and expectations of the rare disease community**, as outlined in its **2022 Position Paper**.

EURORDIS engaged with **EU policymakers**, particularly **MEPs in the ENVI and LIBE Committees**, to highlight the **unique challenges** rare disease patients face regarding **health data access and individual rights**. It also participated in **public consultations, stakeholder discussions, and the EHDS policy process**. As a **member of the EC eHealth Stakeholder Group and the HealthData@EU Pilot project**, EURORDIS provided **critical insights** on the **specific requirements of rare diseases** within the EHDS framework.

Following the **European Parliament and EU Council agreement on the EHDS in March 2024**, EURORDIS developed **training sessions** to equip **patient advocates** with the knowledge needed to **navigate the new**

framework, **maximise its benefits**, and **mitigate potential risks** in their advocacy work.

EURORDIS's efforts focus on ensuring **inclusivity and equity** in Europe's health data landscape, contributing to a **stronger, patient-centred health data ecosystem** for the rare disease community.

EMPOWER

EURORDIS Digital Advisory Group (DAG)

The **EURORDIS Digital Advisory Group (DAG)** played a key role in consultations for the **FACILITATE project**, providing expert patient perspectives on digital health initiatives. The **first mandate of the DAG's ten expert patient volunteers concluded at the end of 2023**.

In **May 2024**, a **Call for Volunteers** was launched to recruit new members. The **new DAG group was approved by the Board of Directors in late 2024**, ensuring continued patient involvement in digital health policy and research.

DAG volunteers bring **diverse expertise** and have actively contributed to projects such as **FACILITATE, Screen4Care, and CHEERS-AI**, strengthening the integration of **patient perspectives in digital innovation** for rare diseases.

PARTNER

FACILITATE (January 2022 – December 2025, IMI2, Project Partner)

FACILITATE is a **four-year project under the Innovative Health Initiative (IHI)**, focused on **returning clinical trial data to participants** and developing a framework for its **secondary use**. EURORDIS plays a **key role** in ensuring the project meets **patient needs** across **ethical, legal, and technical aspects**.

The **EURORDIS Digital Advisory Group (DAG)** has actively contributed to discussions on **ethical frameworks for returning clinical trial data**, **patient consent processes**, and **secondary use of trial data**. These insights have been incorporated into FACILITATE's ongoing work. To enhance awareness, EURORDIS **organised a webinar on 11 December 2024**, titled **"Understanding the Secondary Use of Clinical Trial Data,"** featuring two patient representatives.

Together4RD

Together4RD fosters collaboration between **European Reference Networks (ERNs) and industry** to strengthen **rare disease research** and address **unmet medical needs** for the **95% of rare diseases without dedicated treatments**.

In **2024**, EURORDIS, as a **Steering Group member**, supported a **shift in ERN-industry partnerships** from a **healthcare focus to a research-driven approach**. Key contributions included:

- **Supporting the development of an in-person policy workshop** to align **multi-stakeholder perspectives** on ERN-industry partnerships in research;
- **Initiating a joint collaboration between the Rare Disease Moonshot and Together4RD** to host a **webinar on best practices in public-private partnerships for rare disease research**;
- **Successfully advocating for the creation of a Together4RD Task Force**, which will **scope a proposal for a new Forum for ERN-Industry collaboration**.

DEVELOPMENT AND ACCESS TO DIAGNOSTICS AND THERAPIES

04[05]

"DEVELOPMENT AND AVAILABILITY, ACCESSIBILITY, AFFORDABILITY OF TREATMENTS, PARTICULARLY TRANSFORMATIVE OR CURATIVE THERAPIES – THE GOAL OF 1,000 NEW THERAPIES WITHIN 10 YEARS"

ADVOCATE

Advocating for the implementation of an EU pharmaceutical regulatory and policy framework that addresses the challenges of people living with rare diseases: On EU Pharmaceutical legislation, Orphan Medicinal Products and Paediatrics Regulations

In 2024, EURORDIS concentrated its advocacy efforts on ensuring that the **EU pharmaceutical legislation, Orphan Medicinal Products, and Paediatrics Regulations** address the challenges faced by people living with rare diseases.

Initial efforts focused on the **European Parliament**, which successfully adopted its position on **10 April 2024**, just before the end of its five-year mandate. EURORDIS played a key role in advocating for amendments that were ultimately included in the final position, strengthening the **European Commission's original proposals**. These amendments introduced:

- **Mandatory patient consultation** in the legislative process;
- **Progressive market exclusivity** to incentivise research-intensive entities;

- **Facilitated central procurement** to accelerate access to **orphan medicinal products**;
- **Extension of the PRIME scheme**, enhancing regulatory support for promising therapies;
- **A mandate for the European Commission to develop an EU policy framework for rare diseases**.

EURORDIS welcomed these advancements and issued a **press release**, EURORDIS welcomes Parliament plenary vote on pharma package, which was covered by **EU media**.

Since April, EURORDIS has shifted its focus to the **Council**, engaging with **Member States** as negotiations continue under the **Belgian and Hungarian Presidencies**. A **targeted outreach programme** was launched to strengthen contact with **key attachés in Brussels**, ensuring **rare diseases remain a priority** in ongoing discussions.To further support its advocacy, EURORDIS has:

- **Organised information meetings** for members, particularly **National Alliances**, to discuss the **European Commission's proposal, key debates, and parliamentary amendments**;
- **Collaborated with National Alliances** to monitor **national-level debates** in EU Member States and provide input for national advocacy efforts;
- **Attended and monitored key EU-level discussions** on pharmaceutical legislation to ensure rare diseases remain a central focus.

Accelerating Clinical Trials in the EU (ACT EU)

The **ACT EU** initiative, supported by the **European Commission, HMA,** and **EMA**, aims to bolster the **EU** as a leading hub for innovative clinical research. Discussions covered **complex trials, paediatric trials, pragmatic trials, digital endpoints, patient-centricity,** and **decentralised trials**, involving experts from **patient organisations, regulatory bodies, HTA agencies, industry,** and **academia**.

In 2024, EURORDIS took part in:

- The ACT EU Clinical Trials Analytics Workshop (25–26 January) on using data from clinical trial registries;
- ACT EU Multistakeholder Platform Advisory Group meetings (20 March, 4 July, 27 September);
- The ACT EU annual progress meeting (22 October), where EURORDIS representative Russell Wheeler shared his views on clinical trial progress in the EU;
- CTR Collaborate Stakeholders meeting (11 September).

ACT EU now provides consolidated advice on clinical trials and the use of the **Clinical Trial Information System**. All meetings discuss the current landscape for clinical trials in Europe. Other key topics include the progress of the **CTR Collaborate project** (involving **Member States** and **ethics committees**) and **CTCG action on patient involvement**. The **COMBINE programme**, which supports clinical trials for both medicines and medical devices, was also introduced.

Lastly, **ACT EU** serves as a platform to address the **EU's political drive for clinical trials**, in collaboration with the **WHO**, which

presented its global guidance for more effective and equitable clinical trials. The **2023 workshop report** is available online.

Advocacy for EU Cooperation on Health Technology Assessment (HTA)

EURORDIS continued advocating for the EU Cooperation on HTA through its HTA Task Force. The Task Force focused on treatment assessment, pricing, and reimbursement across Europe, advising on HTA methodologies and patient engagement. This effort included sharing insights on national HTA systems, expert opinions, and connecting with the scientific community, enhancing EURORDIS' contributions to EU-level HTA cooperation.

Advocating for improved access to treatment for rare diseases

EURORDIS tirelessly advocated for improved access to authorised therapies for rare diseases across the **EU** in 2024. Notably, the **European Commission's EU4Health programme** introduced a call for **Member States** to support structured cooperation in **pricing and reimbursement policies**.

In 2023, EURORDIS escalated its advocacy to **WHO Europe**, participating in the **Novel Medicines Platform (WHO NMP)** initiative, which aims to improve affordable and equitable patient access to effective, novel, high-cost medicines. EURORDIS was an active member of various **Working Groups** on solidarity and sustainability, bringing its expertise in collaborative mechanisms to promote early multistakeholder dialogue and knowledge exchange.

Additionally, through its advocacy related to the Pharmaceutical Package, EURORDIS proposed amendments supporting centralised European procurement for centrally authorised Orphan Medicinal Products (OMPs) and underscored the benefits of early and structured dialogues through the Mechanism of Coordinated Action (MoCA). EURORDIS also advocated for a Joint Action on Pricing and Reimbursement (P&R) and will participate as an associate partner in the work package addressing orphan drug P&R challenges.

EURORDIS continues its involvement in established initiatives, such as the Expert Group on Orphan Drug Incentives and RWE4Decisions, as well as its participation in European Commission-led and EU Council Presidency high-level conferences.

In 2024, EURORDIS presented its approach to international access to orphan medicinal products at a conference in Shanghai, as other regions, including China, have significantly increased investment in rare disease research and development over the past five years.

Ensuring a balance between sustainability and innovation requires new payment models. The HTx project, in which EURORDIS participated, provided key insights into innovative health technology assessment (HTA) approaches, particularly relevant for Central and Eastern European Member States. The upcoming EU Cooperation on HTA, which will begin assessing medicines in February 2025, represents a significant step in advancing access to therapies. It will also create opportunities for discussions on economic methodologies as part of voluntary cooperation.

As part of its engagement in these discussions, EURORDIS organised an ERTC workshop in 2024 to prepare for the implementation of HTA cooperation. The workshop explored different models for estimating fair pricing of orphan medicinal products, reflecting the growing need

for sustainable and equitable pricing frameworks.

Advocating for the harmonisation of Compassionate Use (CU) - Early Access and/or a European fund for CUP

Compassionate Use Programmes (CUPs) are critical for providing innovative medicines to patients, particularly those in urgent need. Since adopting a position on compassionate use for medicines in rare diseases in 2017, EURORDIS has actively contributed to efforts aimed at improving CU in Europe.

In 2022, EURORDIS supported the European Commission's proposal for pharmaceutical legislation revision, advocating for an extension of **Article 83 of Regulation 726/2004** to either establish a European-wide scheme or facilitate an Early Access programme similar to France's, which remains one of the most effective in the EU.

Compassionate use should be systematically discussed when **Community Advisory Boards** meet with industry, and training on compassionate use is essential. It is now included in the **Open Academy** curriculum.

As not all Member States share the same level of information on the benefits of compassionate use and early access, and many patient advocates remain unaware of the potential of these access schemes, EURORDIS decided to explore the creation of an **IHI consortium** to:

- Demonstrate the value of compassionate use and early access;
- Identify barriers and facilitators;
- Establish best practices.

The consortium proposal was presented at an **IHI Brokerage Event** in Brussels from 12–14 November.

EMPOWER

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EURORDIS maintains strong engagement with the **European Medicines Agency (EMA)** through representation in key **EMA Committees and Working Parties**, including the **Committee for Orphan Medicinal Products (COMP), Paediatric Committee (PDCO), Committee for Advanced Therapies (CAT), Patients' and Consumers' Working Party (PCWP), Pharmacovigilance and Risk Assessment Committee (PRAC), Scientific Advice Working Party (SAWP), and the Committee on Herbal Medicinal Products (HMPC)**. EURORDIS ensures that **patient perspectives** are integrated into EMA discussions, supporting the participation of rare disease patients in regulatory processes.

EURORDIS Therapeutic Action Group (TAG) Coordination

The **TAG** consists of **13 members** representing key EMA committees and EURORDIS. In **2024**, **11 monthly calls** were held, focusing on the **EU pharmaceutical legislation**, the **Orphan Medicinal Products (OMP) & Paediatric Regulations**, patient data generation, and **patient involvement at the EMA**. TAG members also participated in the **Black Pearl Awards & ERTC (February and October 2024)**. In line with EURORDIS' volunteer policy, the **TAG terms of reference were updated in 2024**.

Committee for Orphan Medicinal Products (COMP)

EURORDIS issued a **letter of support for three patient representatives** and endorsed a **candidate for the European Commission's call for Civil Society representatives**, who was formally appointed. EURORDIS also **assisted the three appointed patient representatives** in their **integration into COMP (July 2024)**. At the **Strategic Learning Review Meetings (SLRM)**, EURORDIS organised a session on **(High) Unmet Medical Needs in Leuven (March 2024)** and an expert presented at the **Budapest session (October 2024)**. EURORDIS also contributed to **COMP working groups**, particularly through the **CollaboRARE pilot**, which was presented to the **EMA Patients' and Consumers' Working Party (PCWP) and the EMA/HMA Big Data Stakeholder Forum,** highlighting the joint efforts of EURORDIS and EMA to integrate patient experience data in regulatory processes.

Committee for Advanced Therapies (CAT)

EURORDIS supported the **rare disease patient representative (alternate) appointed in July 2022**, who actively participates in **monthly plenary meetings** and attended the **SLRMs in Leuven (March) and Budapest (October 2024)**. EURORDIS also **supported an application for the next CAT mandate**.

Paediatric Committee (PDCO)

EURORDIS provided ongoing monthly support to the four rare disease patient representatives on the PDCO (two members and two alternates). Additionally, EURORDIS supported their participation in the EMA Strategic Learning Review Meetings held in Leuven (March 2024) and Budapest (October 2024).

Pharmacovigilance Risk Assessment Committee (PRAC)

EURORDIS continues to support the **two rare disease patient representatives (member and alternate)** appointed to PRAC in **June 2022**, ensuring their participation in **monthly plenary meetings and the 2024 SLRMs in Leuven and Budapest**. EURORDIS also **supported an application for the next PRAC mandate**.

Scientific Advice Working Party (SAWP)

EURORDIS identified, mentored, and supported **21 patient experts (84%)** to participate in **scientific advice and protocol assistance activities**. In **2024**, EURORDIS reviewed **131 dossiers**, with **25 requiring patient input**, ensuring that patient perspectives are incorporated into **scientific advice groups (SAGs) and regulatory discussions**.

Committee for Medicinal Products for Human Use (CHMP)

EURORDIS remained engaged in **CHMP Early Dialogues**, contributing to **six product assessments** when no other rare disease organisation could respond. **Six memos** were submitted to **CHMP**, summarising insights from **11 patients across 10**

European countries.

Following the **non-renewal of the conditional marketing authorisation for Translarna® (Duchenne Muscular Dystrophy)**, EURORDIS **proposed a webinar** for patient organisations to explain the CHMP's reasoning. EURORDIS also participated in the **EMA webinar on 10 December 2024**.

Management Board (MB)

A **EURORDIS staff member** participated in **four plenary meetings** and **two ad hoc meetings** on EMA independence policies and served as chair of the **Management Board Audit and Risks Group (MBARG)**. As the **civil society mandate ends in June 2025**, EURORDIS has **submitted an application for a further term**.

Patients' and Consumers' Working Party (PCWP)

EURORDIS ensures patient perspectives are integrated into **PCWP discussions**. **François Houÿez and Russell Wheeler (Leber Hereditary Optic Neuropathy UK Society)** serve as alternate members, with EURORDIS **actively participating in all 2024 meetings**.

Key contributions include:

- Input into the **EMA Reflection Paper on Patient Experience Data**.
- Membership in the **ACT-EU Multistakeholder Platform and its Advisory Group**.
- Consultation on the **European list of critical medicines**.
- Presentation of EURORDIS' **DITA Task Force analysis on Medicines Overviews**, covering **179 CHMP opinions (2019-2023)**.
- Participation in a **DG SANTE/HaDEA conference on hospital exemptions**.

Through its extensive involvement in **EMA committees and working groups**, EURORDIS continues to **ensure the voice of rare disease patients is integrated into European regulatory decision-making**.

Additional activities

EURORDIS continues to contribute to the **review of EMA public information documents**, ensuring accessibility and clarity for patients. When no specific patient group exists for a disease in the EMA database, EURORDIS steps in to review **Medicine Overviews, Package Leaflets, and Q&A documents**. In **2024**, EURORDIS reviewed **three Medicine Overviews and three Package Leaflets**.

EURORDIS also produces a **Monthly Therapeutic Report**, providing key updates on regulatory developments and therapeutic advancements. In **2024**, **11 reports** were published and distributed to **around 500 stakeholders**, including **patients, regulators, and clinicians**. Summaries were also featured in the **EURORDIS Member News** each month, ensuring broad access to critical therapeutic information.

Supporting patient involvement in Health Technology Assessment (HTA) activities: EURORDIS Task Force on HTA

The **EURORDIS Task Force on HTA** consists of **eight volunteers from member organisations across the EU** who provide expertise on **assessment, pricing, and reimbursement of treatments**. The task force actively engages in **policy debates, public consultations, and discussions on national HTA systems**.

In **2024**, the task force focused on shaping **EURORDIS' responses to consultations**

on the HTAR Implementing Acts, with key contributions including:

- **Gathering members' expertise and opinions** to consolidate EURORDIS' positions on the HTAR Implementing Acts;
- **Initiating a review** of the **HTA and reimbursement status of specific drugs**;
- **Providing feedback and inputs** on EURORDIS-led projects, strengthening policy positions;
- **Contributing to flash debates** at the **European Roundtable of Companies (ERTC)**;
- **Holding a face-to-face meeting in Paris (May 2024)** to discuss HTA priorities and advocacy efforts.

Supporting patient involvement in dialogue with payers through the Mechanism of Coordinated Access to Orphan Medicinal Products (MoCA)

EURORDIS plays a key role in **MoCA**, facilitating **patient and clinician engagement** in discussions on **access to orphan medicinal products**. In **2024**, EURORDIS contributed by:

- **Identifying and supporting two rare disease patients and one ERN clinician** to participate in **MoCA meetings**;
- **Participating in four MoCA Steering Committee meetings**, ensuring rare disease perspectives were represented in **multi-stakeholder actions**;
- **Organising a MoCA webinar with EUCOPE** to discuss **policy and access challenges**;
- **Presenting on MoCA at key events**, including the **ERTC, World Orphan Drug Congress Europe, and WHO Novel Medicines Platform**;
- **Disseminating key research**, including the publication of Patients, Payers, and Developers of Orphan Medicinal Products: Lessons Learned from 10 Years' Multi-Stakeholder Dialogue on Improving Access in Europe via MoCA.

Supporting patient involvement in developing quality information on medicines: Drug Information, Transparency and Access Task Force (DITA TF)

The **DITA Task Force** met **eight times in 2024**, including a **face-to-face session in Paris (21 May)**, focusing on **regulatory engagement and patient involvement in pharmaceutical policy.**

Key contributions included:

- **Active participation in EMA and ACT EU workshops on clinical trials, patient registries, AI, and real-world evidence;**
- **Joining the ACT EU Multi-Stakeholder Group**, ensuring alignment with EU initiatives;
- **DITA Task Force representation in the ACT EU Annual Meeting Programme Committee;**
- **Reviewing the EMA's scientific advice activity report**, assessing patient involvement and expert remuneration policies;
- **Providing feedback on early results from the EMA Raw Data Pilot Project.**

Policy contributions in **2024** focused on:

- **Advocating for patient representation as full members of the CHMP** during the **revision of pharmaceutical legislation;**

- **Contributing to the EMA Reflection Paper** on efficacy assessments based on **single-arm trials;**
- **Reviewing EMA's approach to real-world data** in non-interventional studies.

PARTNER

EUCAPA – HTA training (EU4Health, March 2023-March 2025, Project Lead)

EUCAPA is a training initiative designed to prepare patients and patient representatives for meaningful involvement in health technology assessment (HTA) ahead of the EU HTA Regulation (EU) 2021/2282 (HTAR), which takes effect in January 2025. The project ensures that patients and patient organisations understand **HTA processes**, equipping them with the necessary **knowledge and skills** to engage in **scientific consultations and assessments** under the new regulation.

EURORDIS **leads the EUCAPA project**, coordinating both **WP1 (Project Management)** and **WP2 (Training and Content Development)**. WP1 focuses on **project coordination, data governance, quality management, and ethics**, while WP2 develops training **curricula and materials** through a **co-creation process with project partners**. Training is offered at three levels – **introductory, fast-track, and extended** – ensuring accessibility to a broad range of patient advocates.

Training sessions launched at the **end of 2023**, with the **Introductory Training now available online**. In **2024**, two **Fast-Track Training** sessions took place in **February**, followed by a **third in April**, all receiving **highly positive feedback**. The first **Extended Training** was held from **5–7 March at UMIT TIROL in Hall in Tirol, Austria**, with **10 participants**. A second **in-person session** took place from **11–13 June**, followed by an **online session in October**. Additional training sessions are planned, with content **continuously updated** to reflect changes in the **HTAR Implementing Acts**.

EUCAPA has engaged participants from **26 European countries**, including **Albania, Austria, Belgium, France, Germany, Italy, Spain, and the UK**, as well as **seven non-European countries** such as **Argentina, China/Hong Kong, Kenya, Philippines, South Africa, Turkey, and the USA**.

To maximise its reach, EUCAPA's **communication strategy** includes **regular newsletters, participation in key events such as the HTAi Conference, and targeted outreach to patient communities**. At the **HTAi Conference**, EURORDIS contributed to a **panel discussion** on "How Can Meaningful Patient Engagement in Health Technology Assessment Help Shape More Sustainable and Fair Healthcare Systems?". Additionally, the project **identified 37 diseases** likely to be assessed under **HTA Regulation** due to their **potential for Advanced Therapy Medicinal Products (ATMPs)**.

More Effectively Using Registries to suppOrt PAtient-centred Regulatory and HTA decision-making' (More-EUROPA, 2023-2028, Horizon Europe, Project Partner)

More-EUROPA aims to enhance the integration of Real-World Evidence (RWE) into regulatory and HTA decision-making by developing and implementing standards that meet the evidentiary requirements of European authorities. The initiative focuses on **improving the development, registration, and assessment of medicinal products** by ensuring that RWE is efficiently and reliably utilised in decision-making processes.

On **12 February 2024**, the **More-EUROPA team participated in a Patient Registries Workshop at the EMA**. Discussions focused on **the EMA qualification procedure for patient registries**, aiming to clarify its **benefits, address current limitations, and propose measures to optimise the process**.

On the second day, the workshop further explored **the value and application of patient registries in regulatory decision-making**, identifying contexts in which **registry data are 'fit for purpose'** and assessing **tools to improve data discoverability and evaluation**. Through these discussions, **More-EUROPA contributes to strengthening the role of RWE in shaping future regulatory and HTA frameworks across Europe**.

Next Generation Health Technology Assessment (HTx, January 2019-June 2024, Horizon 2020, Project Partner)

HTx came to an end in 2024. It aimed to develop a framework for Next Generation Health Technology Assessment (HTA) to support patient-centred, societally oriented, and real-time decision-making on health technologies across Europe. In the final months, the patient toolkit was completed. It includes videos on, for example, the development and use of predictive models, selecting appropriate patient-reported outcomes, and policies and real-world evidence data. It also includes the publication of an article on the extension of the Consolidated Health Economic Evaluation Reporting Standards (CHEERS) to incorporate artificial intelligence in evaluations (CHEERS-AI).

PHOTO: MORE-EUROPA TEAM

GetReal Institute

The GetReal Institute is an independent, multi-stakeholder European forum dedicated to advancing the adoption and implementation of Real-World Evidence (RWE) in regulatory, health technology assessment, and clinical decision-making In 2022, EURORDIS joined GRI as a co-founder and Board member, focusing on reducing barriers to the use of secondary data, bridging the gap between Randomised Control Trials (RCT) and RWE, and meeting the evidence needs of healthcare decision-makers.

In 2023, GRI outlined its objectives and deliverables:

- Establish itself as a key forum for sharing knowledge and prioritising critical opportunities and challenges in RWE;
- Undertake case studies and demonstration projects, and publish research to address scientific and operational uncertainties;
- Develop trusted resources and guidelines for best practices in RWE, filling existing gaps;
- Offer skill development training through partnerships with academic institutions (GetReal Academy).

In 2024, an interim Medical Director was appointed for a period of six months, and the Institute decided to revise its approach for its members to be better involved in all activities. Four working groups were created:

- WG1 – Collaboration & Engagement: Establish an impactful forum for stakeholder collaboration;
- WG2 – Data & Methods: Contribute to the development and analysis of methods and best practices;
- WG3 – Knowledge Dissemination: Identify, develop, and share resources and experiences to facilitate the adoption of RWE;
- WG4 – Education & Training: Build a knowledge-sharing community for skills development in the application of RWE.

The Institute is also a partner in the Sustain-HTA Horizon Europe project, in charge of education activities on RWE.

VACCELERATE (January 2021-January 2024, Horizon 2020, Project Advisor)

VACCELERATE is a European network supporting COVID-19 vaccine trials, led by the University Hospital Cologne, Germany. It brings together **29 national partners from 18 EU member states and five associated countries**, coordinating efforts to enhance **clinical trial preparedness and response capacity**. Due to recruitment challenges, the project duration was **extended by 12 months**, introducing a **new task on Clinical Trials Preparedness**. Current trials focus on **booster vaccinations across different age groups**, with an added **emphasis on long-term follow-up**. On **18–19 January 2024**, a **workshop on Adaptive Platform Trials** was held in **Köln**, discussing **statistical methodologies, expert insights from vaccine trialists, and applications to specific microbes** such as **avian flu, MERS, and West Nile virus**. Regulators also provided **perspectives on the role of platform trials in vaccine development**.

Ongoing **clinical trials** under VACCELERATE include:

- **Booster Vaccination Doses in the Elderly;**
- **Booster Vaccination Doses in Adults;**
- **Vaccination regimen in children aged 12–16 years.**

PHOTO: 2ND REMEDI4ALL MULTI-STAKEHOLDER MEETING, «ENSURING PATIENT CENTRICITY IN OSTEOGENESIS IMPERFECTA RESEARCH.»

REMEDi4ALL (September 2022-August 2027, Horizon Europe, Project Partner)

REMEDi4ALL supports the development and implementation of repurposed medicines to address unmet medical needs, with a strong focus on rare diseases (RD). As the **Work Package (WP) lead on Patients & Users' Engagement**, EURORDIS plays a key role in **ensuring patient-centricity** throughout the **drug repurposing lifecycle**. Key activities include **developing patient engagement processes**, **organising multi-stakeholder meetings**, and **contributing to training and capacity building** within the consortium.

Progress in 2024

In **June 2024**, EURORDIS organised the **second REMEDi4ALL Multi-Stakeholder Meeting**, *"Ensuring Patient Centricity in Osteogenesis Imperfecta Research."* The event attracted **60 in-person and 80 online participants**, and EURORDIS is currently working on a **publication of the meeting outcomes**.

Patient engagement activities advanced through collaboration with **Patient Champions and Patient Advocacy Groups** in **demonstrator projects**, including:

- **Drafting a patient factsheet** to support trial participants;
- **Establishing a Buddy System** to provide **one-on-one peer support** for trial participants;
- **Organising focus groups** to gather patient input ahead of scientific advice meetings with regulators;
- **Developing an onboarding and prioritisation process** to ensure transparency in decision-making for new projects;
- **Creating a REMEDi4ALL Code of Conduct** for patient engagement activities.

Training and Capacity Building

Building on the **training landscape developed in 2023**, REMEDi4ALL launched a **drug repurposing curriculum**, leading to the **soft launch of the Digital Repurposing Academy** in **September 2024**, with a **full launch planned for early 2025**.
In **2024**, EURORDIS also contributed to the:

- **1st REMEDi4ALL Hackathon for early research careers**, featuring patient representatives as speakers;
- **Preparation of the first REMEDi4ALL in-person training for Patient Advocates**, set to take place in **June 2025 in Barcelona**, alongside the **EURORDIS Open Academy** (Repurposing Bootcamp for Patient Representatives).

JOIN4ATMPS (January 2024 - December 2026, HORIZON - CSA, Partner)

JOIN4ATMP aims to accelerate and de-risk the development of Advanced Therapy Medicinal Products (ATMPs) in Europe, ensuring widespread access while fostering a competitive and innovative European Good Manufacturing Practice (GMP) landscape. The project aligns with the **Strategic Call Destination 6**, supporting **an innovative, sustainable, and globally competitive healthcare industry**. Its overarching goal is to **facilitate the translation of ATMPs from preclinical studies to clinical use and market access**.

To achieve this, JOIN4ATMP is mapping the hurdles for clinical applications of ATMPs, identifying real-world solutions from developers and regulators within and beyond the EU, and designing policy and regulatory recommendations to advance ATMP development.

A major research activity within JOIN4ATMP was the development of a questionnaire covering key work package (WP) topics. Initially consisting of over 200 questions, it was refined to approximately 100, with a particular focus on patient leaders active in ATMP development. The final questionnaire, launched at the end of 2024, targets four key stakeholder groups: developers, payers, regulators, and patient leads, each with tailored sets of questions to gather insights relevant to their expertise.

EURORDIS contributed to the project's governance by suggesting members for the **Scientific Advisory Board**, resulting in the inclusion of a **EURORDIS Board Member**. Additionally, EURORDIS played a central role in organising and chairing a dedicated JOIN4ATMP session at the **World Orphan Drug Congress (WODC) 2024**, which brought together representatives from **EUROPABIO, EUHA, and JOIN4ATMP**. The session focused on identifying regulatory and operational hurdles, streamlining development pathways, and strengthening European cooperation between academic and commercial developers. Discussions highlighted the need to cultivate a sustainable pipeline of ATMPs by aligning stakeholder expectations, addressing existing barriers, and co-designing recommendations to reinforce the ATMP ecosystem in Europe.

RealiseD (To start in January 2025 – IHI - Partner)

The ambition of REALISED is to change the paradigm of clinical trial design for rare and ultrarare diseases by providing ground-breaking methodological and operational solutions, designed through a process of co-creation between different stakeholders. This approach increases their acceptability and usability by drug developers. This will be delivered through a tripartite MISSION encompassing:

- Provision of optimized methodological solutions for innovative clinical trial designs and analysis methods based on already developed statistical methods in borderline areas and preparational of the floor for pioneering inference paradigms for drug approval including regulatory considerations;
- Blueprint of the network of qualified clinical research sites, including in the areas of ATMPs, coupled with the predictable referral of patients;
- Acceleration of the operationalisation of the methodological and clinical frameworks through the delivery of co-created playbooks pressure-tested via specific and well-chosen use case studies and smooth integration into the overall rare diseases (RD) ecosystem.

EURORDIS will be leading the work on patient engagement and multi-stakeholder collaboration.

DeCODe (September 2024 – September 2026, SAB member)

EURORDIS is represented in the Scientific Advisory Board of the EC funded project DeCODe which kicked off in Brussels in September 2024. This two-year ground-breaking initiative aimed at catalysing innovation and addressing the unique healthcare needs of people living with a rare disease, specifically children. This collaborative group, comprising clinicians, researchers, industry experts, and regulatory authorities, will develop a platform for developing safe and effective paediatric and orphan medical devices.

Screen4Care (S4C, September 2021 – September 2026, IMI 2, Project Partner)

Screen4Care is developing innovative approaches to expedite rare disease diagnoses by integrating genetic newborn screening and digital technologies. EURORDIS plays a central role in the initiative, leading the **Patient Advisory Board (PAB)** and ensuring that the rare disease community's priorities are reflected across all project activities.

In **2024**, significant progress was made in developing the **ACT Panel list**, a key component of the project. This process involved an **inclusive and multi-stakeholder approach**, incorporating insights from the **Rare Barometer survey results**, a **nomination process**, and **consultations with key experts**, including **clinicians, geneticists, genetic counsellors, policymakers, and patient representatives**. The selection of **54 genes for inclusion in the ACT panel** followed a **systematic, evidence-based approach**, with final guidance provided by the **Screen4Care Scientific Advisory Board (S4C SAB)**. The **final gene list**, along with a **detailed methodology document**, has been made available to stakeholders.

A **workshop** was organised to present **Screen4Care's findings** and facilitate discussions on key issues surrounding **genetic newborn screening**. Topics included **structuring a viable and broadly accepted genetic NBS programme, parents' experiences in genetic NBS pilots, ethical considerations, implementation barriers, and economic and social factors**.

PHOTO: COUNCIL OF NATIONAL ALLIANCES MEETING. PARIS. 2024

International Consortium for Rare Disease Research (IRDiRC)

The International Rare Diseases Research Consortium (IRDiRC) brings together national and international funding bodies, companies, patient advocacy organisations, and researchers to foster global collaboration in rare disease research. Its vision is for all individuals with a rare disease to receive an accurate diagnosis, appropriate care, and available therapy within one year of seeking medical attention.

EURORDIS has been a key member of IRDiRC since its inception in 2011, actively participating in the Consortium Assembly and contributing to numerous activities aimed at achieving IRDiRC's goals. Additionally, EURORDIS staff are actively involved in the Consortium Assembly and the Therapies Scientific Committee, with further contributions from an Open Academy alumnus.

EURORDIS has led or participated in various task forces, including those focusing on Sustainable Economic Models in Repurposing and Drug Repurposing Guidebooks, contributing to publications and resources essential for drug development. Efforts include participation in the Pluto Project on Disregarded Rare Diseases and the Newborn Screening Initiative, with involvement in upcoming publications and workshops.

EURORDIS remains committed to ensuring the sustainability of the Orphan Drug Development Guidebook (ODDG), co-created within the IRDiRC Therapies Scientific Committee, through a Memorandum of Understanding with ERDERA partners.

The Newborn Screening Initiative has concluded, contributing to a policy, ethics, and patient perspectives publication in the Rare Disease and Orphan Drugs Journal. In 2024, EURORDIS successfully proposed a working group on paediatric patient engagement, set to begin in 2025. Additionally, EURORDIS authored a paper for the Rare Disease and Orphan Drugs Journal on structuring the European rare disease movement and co-authored a Lancet issue on access within the rare disease landscape.

PHOTO: AVRIL DALY, EURORDIS' PRESIDENT AT THE ECRD 2024

HOLISTIC CARE

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"INTEGRATED MEDICAL AND SOCIAL CARE WITH A HOLISTIC LIFELONG APPROACH AND INCLUSION IN SOCIETY, WITH A GOAL OF REDUCING THE SOCIAL, PSYCHOLOGICAL, AND ECONOMIC BURDEN BY ONE THIRD"

ADVOCATE

ADVOCATING FOR INTEGRATED CARE

EURORDIS continued to advocate for **holistic, lifelong, and person-centred care** as a fundamental part of the future **EU Action Plan on Rare Diseases**. In 2024, it also began its role as **co-leader** in developing **Guidelines for Case Management for Rare Diseases** within the **JARDIN Joint Action** on integrating **European Reference Networks (ERNs) into national healthcare systems**. A detailed **work plan and timeline** were established for the **two-year implementation phase**, with the **Guidelines set for publication in 2026**.

ADVOCATING FOR QUALITY AND ADEQUATE SOCIAL RIGHTS

EURORDIS collaborated with the **Social Platform** and its members to engage in discussions on initiatives stemming from the **European Pillar of Social Rights**, particularly on **long-term care and the rights of informal carers**.

ADVOCATING TO IMPROVE ACCESS TO DISABILITY AND INDEPENDENT LIVING RIGHTS

As a member of the **European Disability Forum (EDF) and the EU Disability Platform**, EURORDIS advocated for **the implementation of the European Strategy for the Rights of Persons with Disabilities 2021-2030**. Throughout the year, it

PHOTO: EUROPEAN DAY OF PERSONS WITH DISABILITIES 2024

participated in **plenary and subgroup meetings**, highlighting the **barriers faced by people with rare diseases** and advocating for their access to the **European Disability Card and independent living support**.

Following the **approval of the European Directives for the European Disability Card**, EURORDIS informed its members about the **national transposition process** and engaged with **National Alliances** to ensure that people with rare diseases living with disabilities can benefit from the **Card and its entitlements**.

Between **June and September**, EURORDIS launched its **Rare Barometer Survey** on **the impact of living with a rare disease, focusing on barriers and enablers to independent living and social participation**. The survey, available in **25 languages**, received **10,478 responses from 92 countries**, including **9,591 from 43 European countries**. In total, **1,754 rare diseases** were represented.

Throughout the **final quarter of 2024**, EURORDIS worked on **data cleaning and analysis**, with results set for **release in February 2025**.

EURORDIS participated in major EU disability events, including a **seminar hosted by the Belgian EU Presidency** and the **European Day of Persons with Disabilities**, co-organised by the **European Commission and EDF**. At both events, EURORDIS called for **improved disability assessment processes across EU countries** to ensure rare disease patients can obtain disability recognition and adequate support.

Additionally, EURORDIS contributed to the **European Commission's SG-SANTE advisory group on "Access to Healthcare for Persons with Disabilities,"** sharing insights from the rare disease community and advocating for **specialised healthcare services tailored to people with disabilities**.

ADVOCATING FOR THE MENTAL HEALTH AND WELLBEING OF PEOPLE LIVING WITH RARE DISEASES

In 2024, EURORDIS focused its advocacy efforts on two key objectives: **strengthening the evidence base** to highlight the unmet mental health needs of people with rare conditions and **ensuring these needs are recognised in both rare disease and broader**

mental health policies.

To address existing gaps, the **Partnership Network** conducted a **prioritisation exercise** to define critical areas for a **systematic literature review**, identifying four key topics: **psychological support after diagnosis, psychological support before receiving a diagnosis, the needs of people living with mental health being a primary characteristic or co-morbidity of their rare condition, and tools and approaches specific to rare disease-related mental health support**.

In 2024, EURORDIS prioritised the **systematic review on psychological support after diagnosis**, analysing the **impact of rare diseases on social isolation, chronic uncertainty, and family dynamics**. The review was **completed in December 2024**, with findings now being prepared for **publication in 2025**. In parallel, EURORDIS initiated the **Rare Barometer Survey on Mental Health**, set to further inform policy recommendations.

EURORDIS worked to support **EU Member States in implementing the UN General Assembly Resolution (A/RES/76/132)**, which calls for **national mental health strategies inclusive of rare disease-specific psychosocial support**. A key milestone was the **EURORDIS Outline Position Paper on Mental Health**, advocating for national rare disease action plans to **explicitly acknowledge mental health impacts** and establish **concrete measures to improve psychosocial care** (**Annex 1a and 1b**). The paper also **proposed a blueprint for integrating psychosocial care into rare disease strategies**, laying the foundation for a **Revised Position Paper in 2025**, informed by the **Rare Barometer Survey on Mental Health**.

To elevate mental health as a policy priority, EURORDIS addressed **EU and national policymakers** through an **Open Letter to EU Future Policymakers**, urging action to:

- **Embed psychological support within patient-centred care**, ensuring that medical services for people with rare diseases are **psychologically informed**;
- **Support patient organisations as key providers of peer and community support**, enabling **early intervention and access to preventative psychosocial care**.

EMPOWER

EURORDIS Social Policy Action Group (SPAG)

In January, EURORDIS launched its **new Social Policy Action Group (SPAG)**, to support its work on holistic care and social participation. The group is composed of **20 volunteers from 12 European countries**, who live with a rare disease themselves or are family members of a person with a rare disease.

The group gathered every quarter to discuss key advocacy issues, and its volunteers were engaged in multiple advocacy activities and events throughout the year. Supporting the **Rare Barometer survey** was one of the SPAG's key activities in 2024, with several volunteers being engaged in the Topic Expert Committee, in reviewing the questionnaire and the translations in their respective languages, and in testing the accessibility of the questionnaire.

Launch of the EURORDIS Mental Health & Wellbeing Partnership Network

The **EURORDIS Mental Health & Wellbeing Partnership Network** marked its **first successful year** since its **launch on World Mental Health Day (10 October 2024)**. Over the past year, the network has expanded significantly, bringing together **137 members from 28 countries (19 EU Member States and 9 non-EU countries)**. The network has strengthened ties within the **rare disease community**, engaging **more than 80 experts with lived experience** and their representatives, alongside **60 medical professionals, psychologists, and researchers** from **19 European Reference Networks (ERNs) and other international rare disease centres**.

The network fosters active engagement, with **18 members volunteering as Mental Health Champions**, contributing to **webinars, podcasts, testimonies**, and participating in **the Steering Group or supporting working groups**.

The primary focus was to establish a **robust governance structure** and transition to a **fully operational network**. EURORDIS coordinated **24 meetings** within the network and held **three All Stakeholder Network Meetings**, enabling members to share experiences, track progress, and explore new engagement opportunities.

The **Steering Group**, composed of **16 members (8 clinicians/researchers and 8 patient advocate leads)**, was established to **oversee strategic development, guide annual work plan implementation, and advise on emerging priorities**. The group meets **twice a year** to ensure the network remains aligned with the evolving needs of the rare disease and mental health communities.

EURORDIS launched and coordinated **two dedicated working groups**, focusing on **Population Needs & Survey, Advocacy & Communication** and **Research & Quality of Life**.

- **Population Needs & Survey, Advocacy & Communication Working Group** met **seven times**, leading the **co-creation of an Outline Position Paper**, coordinating communication efforts (including **blogs, podcasts, and webinars**) to raise awareness of the impact of rare conditions on mental health, and contributing to the **scoping of the EURORDIS Rare Barometer Survey on Mental Health**;
- **Research & Quality of Life Working Group** met **five times**, overseeing research activities, conducting **literature reviews**, identifying **best practices, resources, and tools**, and guiding the **co-creation of the Mentally Healthy Toolkit**.

PARTNER

EURORDIS continued to seek opportunities to develop project proposals aimed at addressing key holistic and social participation priorities, notably within the **Social Innovation (EaSI) strand of the European Social Fund Plus (ESF+)** and within the **Citizens, Equality, Rights and Values programme (CERV).**

LIVES (August 2022-Juillet 2025, EJP-RD JTC 2021, Subcontractor)

The social impact of rare diseases is poorly addressed in scientific literature on quality of life, particularly regarding its effects on work, income, and daily life. The LIVES project aims to address this gap by developing a questionnaire that highlights the social and psychological impact of vascular liver diseases, integrating the theory of social determinants of health and ensuring its transferability to other rare diseases.

The project's objective is to advance research on the quality of life (QoL) of people living with a rare disease by creating an instrument that can be applied to other disease contexts and made freely accessible to the scientific community. This tool will provide insights into the overall quality of life of patients with vascular liver diseases, supporting the design of targeted supportive care programmes.

EURORDIS contributed to the design of the questionnaire in collaboration with AP-HP, providing expertise in question formulation. Additionally, EURORDIS reprocessed and reanalysed data from the Rare Barometer surveys '*Juggling Care and Daily Life*' and '*The Diagnosis Odyssey of People Living with a Rare Disease*' to compare their findings with those of the LIVES project. By sharing these survey results with LIVES researchers, EURORDIS has enhanced understanding of the social impact of rare

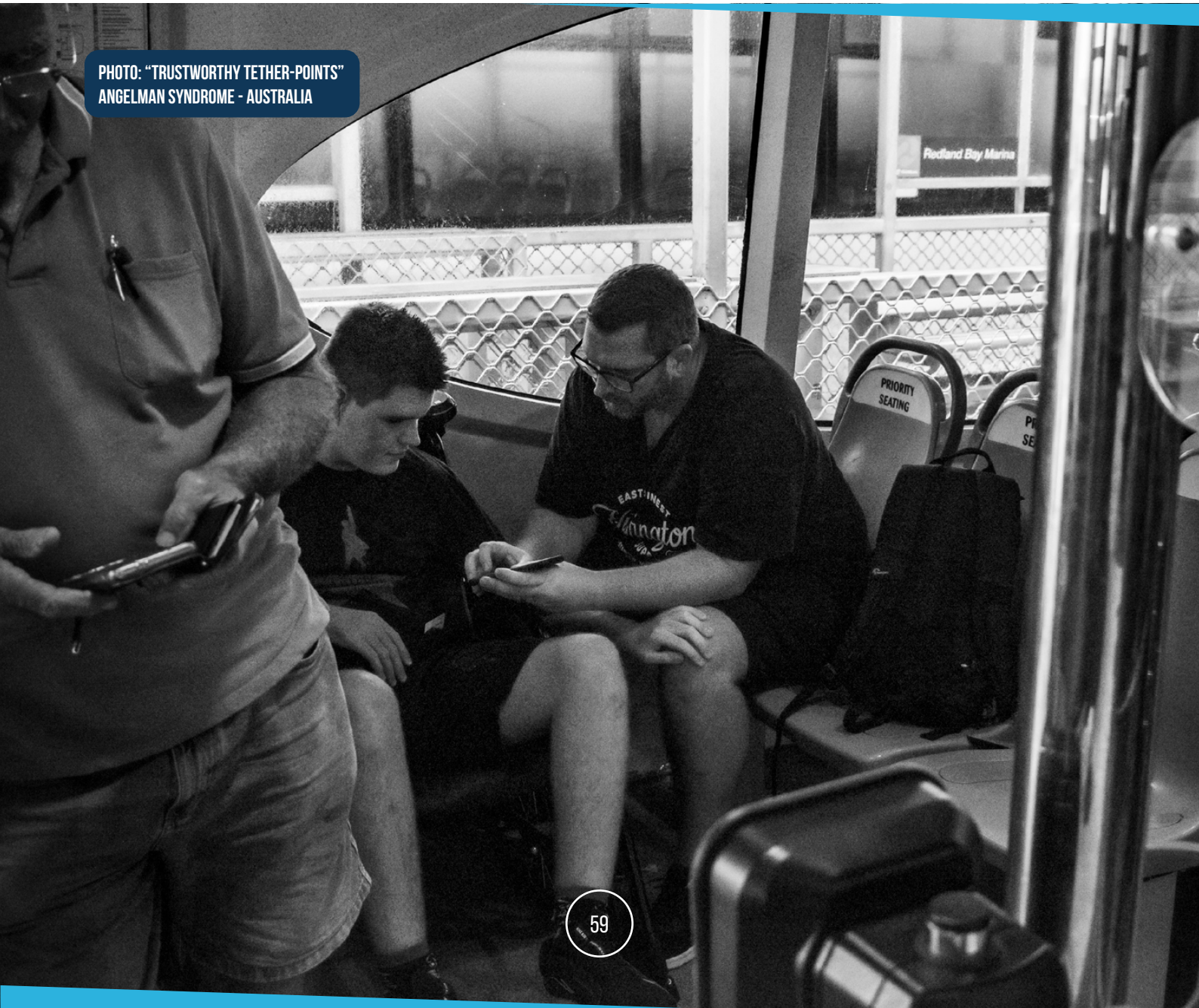


PHOTO: "TRUSTWORTHY TETHER-POINTS"
ANGELMAN SYNDROME - AUSTRALIA

STRATEGIC OBJECTIVE 03:

INCLUSIVE OF ALL RARE DISEASES, ALL REGIONS, "LEAVING NO ONE BEHIND"

By 2030, EURORDIS will have consolidated its scope to "leave no one behind" in the rare disease community by covering:

All therapeutic areas, including genetic or non-genetic rare diseases, and rare cancers, with progress to be made with regard to rare infections and rare health hazards

All countries in geographical Europe prioritising Eastern and Southern Members of the EU, European Economic Area & EU Accessing Countries

All rare disease prevalence and incidence levels, particularly the ones affecting fewer than 1 in 1,000,000

PHOTO: RARE DISEASE DAY 2024

ADVOCATE

RARE CANCERS

Activities with RCAN Members:

RCAN members focused on **raising awareness for rare cancer patients**, advancing **drug development and access**, and ensuring **the integration of rare adult and paediatric cancers into National Cancer Plans (NCPs)**. EURORDIS supported their engagement in **Rare Disease Day**, providing tailored **social media messages** on rare cancers. Key advocacy efforts included **revisions to the EU General Pharmaceutical Legislation (GPL)**, with **three calls** held to discuss **drug development, regulatory improvements, and access inequalities**.

National Cancer Plans (NCPs):

RCAN members identified **gaps in NCPs** regarding rare cancers, which hinder access to specialised healthcare.

To address this, a **sub-working group** was formed to analyse **NCPs across EU Member States and the UK**, aiming to improve patient care by **linking rare cancers with National Rare Disease Plans –** particularly in relation to **orphan medicinal products**. A **draft report was published in December 2024**, with the **final version expected in 2025**.

Collaboration to Raise Awareness:

EURORDIS and RCAN members are working with the **ESMO Rare Cancer Working Group** and the **European Cancer Organisation (ECO)** to elevate rare cancer issues at the **European level**. This includes **conference participation** and engagement in **EU public consultations** to advocate for better policies and support for rare cancer patients.

VERY RARE AND ULTRA-RARE DISEASES

EURORDIS has prioritised policy initiatives and advocacy efforts to address the challenges faced by individuals with **very rare and ultra-rare diseases**. This includes contributions to the **revision of the EU Regulation on Orphan Medicinal Products**, participation in **projects like SOLVE-RD** (focused on undiagnosed diseases) and **Screen4Care** (promoting early diagnosis and newborn screening).

Additionally, EURORDIS has actively engaged with **EMA Committees**, advocating for **access to therapies for very rare diseases and advanced treatments**. The organisation also supports the provision of **highly specialised services through European Reference Networks (ERNs)**, ensuring that patients benefit from expertise at the EU level.

EMPOWER

Launch of the Rare Cancer Advocates Network (RCAN)

RCAN was inaugurated in January 2023, assembling rare cancer ePAGs from four ERNs related to rare cancers – PaedCan (paediatric cancers), **EURACAN** (rare adult solid cancers), **EuroBloodNet** (rare haematological diseases including rare adult blood cancers), **GENTURIS** (genetic tumour risk syndromes), alongside ePAGs in the "endocrine tumours" sub-network of ENDO-ERN. Dorica Dan, a EURORDIS Board member, Chair of the Romanian association for rare cancers and the Romanian national alliance for rare diseases, also contributes to the network. Governed by the ePAGs Constitution and Rules of Procedure, **RCAN started with 33 ePAGs, expanding to 38 by December 2024.**

RCAN is distinct in uniting rare cancer patient advocates/ePAGs across all cancer types and ages at a national, European, and international level, fostering extensive outreach to patients and caregivers. This collaboration is further enhanced by connections with various societies, organisations, and networks such as **WECAN, the Rare Cancers Working Group of ESMO, SIOPe, EHA PAC, and ECO PAC:**

- WECAN – Workgroup of European Cancer Patient Advocacy Networks;
- Rare Cancers Working Group of the European Society of Medical Oncology (ESMO);
- European Society for Paediatric Oncology (SIOPe;)
- European Hematology Association Patients Advocacy Committee (EHA PAC);
- European Cancer Organisation Patients Advisory Committee (ECO PAC).

PARTNER

Rare Disease Day 2024

The **17th edition of Rare Disease Day** saw **over 1,000 events** held in **100+ countries**, with **Bulgaria, Ecuador, Nepal, Rwanda, and Thailand** joining the campaign. Events ranged from **political conferences and medical symposia to sports activities and community gatherings**. Branded materials were widely downloaded, and **key infographic cards were translated into seven additional languages** upon request. The **#RareDiseaseDay challenge** encouraged global participation through themed daily social media posts. Despite energy constraints, **monuments, homes, and offices worldwide were illuminated** as part of the **Global Chain of Lights**, showing solidarity with the rare disease community.

RDD maintained a strong **media presence**, with engagement from **policymakers,**

researchers, medical professionals, and patient advocates worldwide. The **Rare Disease Day website (rarediseaseday.org)** remained a key platform for campaign materials and event listings, with a **redesigned menu improving navigation**.

To support participation, EURORDIS hosted a **capacity-building webinar**, focusing on **amplifying young voices and strengthening National Alliances' role in the campaign**. The **Outreach Group**, composed of communication experts from National Alliances, held **four meetings**, while the **Steering Committee**, representing the most active alliances, met **twice**.

The campaign emphasised **diversity and inclusion**, featuring a broad range of therapeutic areas, regions, and underrepresented groups, including **rare cancers and undiagnosed patients**. A new **Rare Disease Day Young Ambassadors group** was introduced to engage young advocates. The **Rare Disease Day on Air podcast** aired **nine episodes** from **January to 29 February 2024**, sharing personal stories of resilience and advocacy. RDD 2024 achieved a **global digital reach of 89 million people**, with **46,000 mentions and 1.1 million interactions** across platforms. The **official campaign video** received **positive feedback** from National Alliances, with **68% finding it helpful, 88% engaging, and 76% representative**. A total of **1,803 campaign-related videos** were produced and shared worldwide.

RDD continued to be a powerful advocacy tool, with **60% of National Alliances leveraging the campaign to drive policy initiatives**. Engagement in the **Light Up for Rare movement** increased to **72%, up from 59% in 2023**. A **webinar on youth engagement** further reinforced the campaign's commitment to involving younger generations.

The campaign's impact was widely recognised, with **66% of the general public and 84% of National Alliances confirming that RDD effectively supports their objectives**. Planning for **RDD 2025** is already underway, with a focus on expanding awareness, strengthening advocacy, and increasing global participation.

PHOTO: NATIONAL CHILDRENS SPECIALISED HOSPITAL OCHMATDIT, UKRAINE. RARE DISEASE DAY 2024

CROSS-CUTTING PRIORITIES:

TO SUPPORT OUR 3 STRATEGIC OBJECTIVES

COMMUNICATION AND DISSEMINATION

PEOPLE (STAFF AND VOLUNTEERS)

GOVERNANCE

RESOURCE DEVELOPMENT AND SUSTAINABILITY

PHOTO: ZAINAB ALANI, FEATURED PODCAST GUEST IN 2024

COMMUNICATION AND DISSEMINATION

In 2024, EURORDIS advanced its **communication strategy**, aligned with the **Strategic Objectives 2021–2030**, to support advocacy and engagement. The strategy is built on four pillars: **Consistency** (strengthening EURORDIS' image), **Credibility** (positioning as a leading voice in EU policy), **Connectivity** (fostering inclusive engagement), and **Community** (leveraging networks to strengthen the rare disease movement).

Podcast: The **Rare on Air podcast** provided a platform for patient advocates and individuals affected by rare diseases. EURORDIS released **16 episodes**, exceeding its planned **12**, featuring discussions on **ultra-rare conditions, diagnostic delays, and advocacy through sports**. The podcast reached **1,561 listens** and gained **306 followers** across platforms.

Newsletters: EURORDIS' **monthly eNews** welcomed **556 new subscribers** in 2024, with an **average open rate of 29.73%**, slightly above the **non-profit sector average**, though click rates dropped. Key topics included **HTA 2025, Rare Disease Week, and emerging EU priorities in rare disease research**. The **bi-monthly Member News**, available in **six languages**, issued **22 editions**, ensuring continued engagement with member organisations.

Website and digital performance: EURORDIS improved its website's **speed and accessibility**. Key **advocacy pages were updated in six languages**, expanding reach. Website engagement **exceeded expectations**, with a **92.02% engagement rate** and visitors from **209 countries**. A **43-page audit report** provided recommendations for improvement in 2025.

Reference documents and translations: EURORDIS ensured **accessibility and clarity** in key publications, including the **Activity Report 2023 and Work Programme 2024**, prioritising **screen-reader-friendly formatting, alt text, and strong visual contrast**. Additionally, all priority **advocacy web pages were translated into six languages**, enhancing accessibility.

Community engagement and media outreach: Monthly **brand mentions** reached **2.8 million**. EURORDIS was cited **48 times** in major European media, including **Euronews, Politico Europe, Euractiv, and The Parliament Magazine**.

Accessibility initiatives: EURORDIS partnered with **Funka Foundation** for a **website accessibility audit**, identifying key barriers and providing training on **creating accessible PDFs, presentations, and newsletters**.

PHOTO: "10 MINUTES WITH..." LIVESTREAM SERIES

PHOTO: SOFIE SKOUBO, PARALYMPIAN AND RARE DISEASE RESEARCHER. AT THE ECRD 2024

PEOPLE (STAFF AND VOLUNTEERS)

02 04

EURORDIS STAFF

The number of full-time equivalents (FTE) was 49.17 in 2024, down from 51.18 in 2023. At year-end, the team comprised 50 staff members across seven countries: France (30), Spain (10), Belgium (6), United Kingdom (1), Italy (1), Germany (1), Romania (1).

No new positions were created in 2024. Six positions were discontinued:

- Ukraine Response Programme Manager, Hanna Boiko, Brussels;
- Accounting Manager, Annie Rahajarizafy, Paris;
- Events Director, Sharon Ashton-Sirot, remote (UK);
- Operations Junior Manager, Rahul Waslekar, Paris;
- Web Technology Junior Manager, Imène El Aini, Paris;
- Communications Junior Manager, Lise Pernin, Paris.

EURORDIS VOLUNTEERS

EURORDIS volunteers are experts on rare diseases. Most of them are either people living with a rare disease or their caregivers, usually family members. Those not directly impacted by a rare disease are deeply involved in a rare disease patient organisation. EURORDIS' volunteers are committed to providing their expertise and time on specific areas on a voluntary basis. They greatly contibute to raising awareness of rare disease patients' needs and challenges to order to foster research on rare diseases and improve equal access to a timely and accurate diagnosis and adequate available treatments and medicines.

All volunteers adhere to the EURORDIS Charter of Volunteers, adopted on 8 May 2014 during the EURORDIS General Assembly in Berlin. This Charter outlines the values of EURORDIS, the commitments of the volunteers, and EURORDIS' obligations towards them. An internal procedure for volunteer management to standardise processes across all teams was adopted in 2023.

EURORDIS volunteers are selected through a Call for Expression of Interest, based on strict criteria, including a commitment to rare disease advocacy, proficiency in English, and a substantial track record in advocacy. This rigorous selection ensures that EURORDIS consistently proposes qualified candidates for EMA and European Commission committees, often resulting in nominations by the European Commission.

In 2024, EURORDIS had 72 volunteer patient advocates involved in various groups or task forces, with some participating in multiple groups.

TAG (Therapeutic Action Group)

TAG consists of 7 volunteers who represent patients on the European Medicines Agency's scientific committees, engaging significantly in monthly multi-day meetings.

DITA Task Force (Drug Information, Transparency, and Access)

In 2024, 15 trained volunteers contributed to the DITA Task Force's initiatives, supporting EURORDIS representatives in EMA Scientific Committees and Working Parties.

HTA Task Force (Health Technology Assessment)

Comprising 8 volunteers and two coordinating staff, the HTA Task Force advises on HTA policies and shares insights on national assessments and the evolving European Cooperation on HTA.

DAG (Digital and Data Advisory Group)

The 13-member DAG in 2023 advised on digital strategies and policies impacting EURORDIS and its constituents.

SPAG (Social Policy Action Group)

The SPAG consists of 20 volunteers from 12 countries and supports initiatives to enhance access to holistic care and social and human rights for those with rare diseases.

GOVERNANCE

03 04

EURORDIS GOVERNANCE BODIES

The **EURORDIS Annual General Assembly** was held online on 22 May 2024. EURORDIS' full members voted on the Activity and Financial Reports for 2023, as well as the Work Programme and Budget for 2024. Members also voted on the vacant positions for the **Board of Directors (BoD)**. The following candidates were successfully re-elected to the BoD:

- **Avril Daly**, Retina International;
- **Alexandre Méjat**, AFM-Téléthon;
- **Anna Arellanosová**, Rare Diseases Czech Republic;
- **Simona Bellagambi**, UNIAMO.

The Board also extended a warm welcome to new members:

- **Johan de Graaf**, Dutch Pituitary Foundation;
- **Tetiana Kulesha**, Rare Diseases of Ukraine.

Board members were elected for a full mandate of three years. One seat was open for a one-year mandate (Tetiana Kulesha).

The Board of Officers (BoO) is elected annually by the BoD following the General Assembly. In May, the BoO was elected as follows:

- **President**: Avril Daly, Ireland;
- **Vice Presidents**: Dorica Dan, Romania; Simona Bellagambi, Italy;
- **General Secretary**: Geske Wehr, Germany;
- **Deputy General Secretary**: Anna Arellanesová, Czech Republic;
- **Treasurer**: Alain Cornet, Belgium.

The EURORDIS **Core Leadership Team (CLT)** prepares and executes strategies, ensures day-to-day decision-making, and includes the Chief Executive Officer (CEO), Chief Financial Officer (CFO), Chief Operating Officer (COO), Public Affairs Director & Head of European Advocacy, Human Resources Director and People Team Lead, and Governance Senior Manager. The CLT holds weekly meetings to address various strategic, management, and operational issues, facilitating progress towards fulfilling EURORDIS' missions and activities.

EURORDIS KEY PARTNERSHIP WITH THE AFM-TÉLÉTHON

EURORDIS and its co-founder and primary supporter, AFM-Téléthon, have worked on renewing their multi-annual partnership agreement for 2025 onwards.

This agreement facilitates ongoing interactions and collaborative projects within the AFM-Téléthon ecosystem.

KEY PARTNERSHIPS WITH INTERNATIONAL ORGANISATIONS (MOUS)

EURORDIS has developed partnerships with several European and international not-for-profit organisations to work on transversal issues relevant to patients affected by rare diseases. Staff and EURORDIS volunteers engage in various activities depending on the level and type of involvement with international NGO partners.

EURORDIS continues to work with several international organisations in the context of organising Rare Disease Day. Additionally, **EURORDIS has partnerships with several learned societies**, which are listed under the "External Representation" webpage and detailed in our Work Programme 2024.

RESOURCE DEVELOPMENT AND SUSTAINABILITY

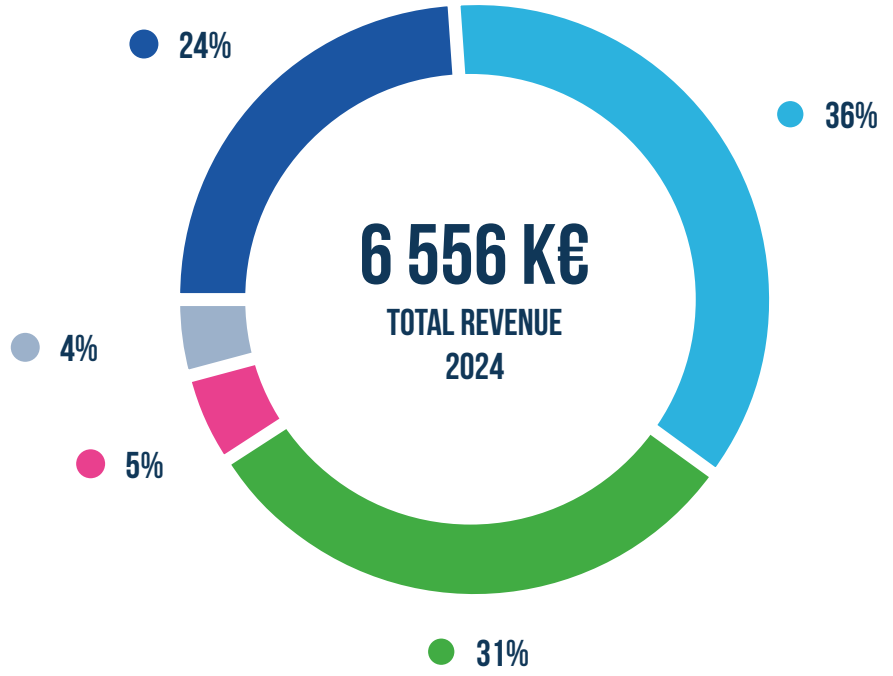
04

In 2024, EURORDIS saw continued engagement and sustained revenue across its resource development activities:

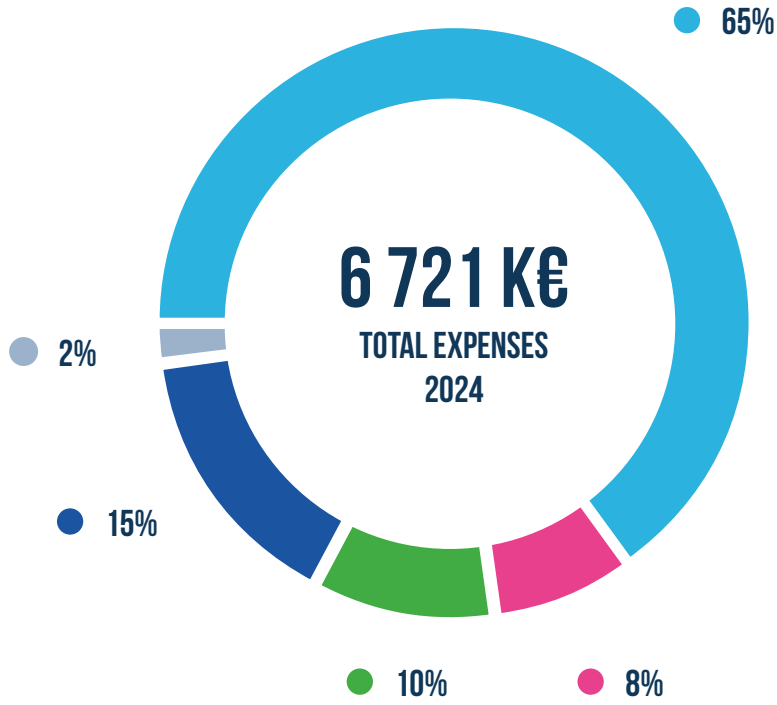
- The EURORDIS Round Table of Companies convened for two key workshops:
  - "Transforming Rare Disease Foresight into Action" in Brussels in February, with 128 attendees including 78 from 44 ERTC member companies.
- "Bridging Perspectives: Preparing for Success in Joint Clinical Assessments in EU HTA" in Barcelona in October, attended by 103 participants, including 71 from 43 ERTC member companies;
- Four webinars for corporate members included topics on the EURORDIS Mental Health programme, the results from the newborn screening Rare Barometer survey, introducing Virginie Bros-Facer as the new CEO of EURORDIS and corporate involvement in Rare Disease Day;
- Fundraising from foundations saw sustained support in 2024, supporting programmes such as Rare Disease Week, patient engagement with the ERNs, patient health data initiatives, and Rare Disease Day;
- A total of 71 health sector corporations supported EURORDIS, including nine new corporate donors.

PHOTO: "THE EYES BELIEVE THE COMPLEXITY"
ANGELMAN SYNDROME - AUSTRALIA

REVENUES AND EXPENSES 2024



PATIENT ORG. AND VOLUNTEERS
EUROPEAN COMMISSION
HEALTH SECTOR CORPORATES
FOUNDATIONS AND NPOS
OTHERS



STAFF COSTS
VOLUNTEERS
LOGISTICS
SERVICES
OTHERS

PHOTO: "UNCONDITIONAL LOVE," TYPE 3 MUCOPOLYSACCHARIDOSIS OR SANFILIPPO SYNDROME. RUSSIA

BOARD OF DIRECTORS

MAY 2024-MAY 2025

PRESIDENT

MS AVRIL DALY
Retina International
Ireland

DIRECTORS

MS ANNA ARELLANESOVA
Rare Diseases Czech Republic
Czechia

MS SIMONA BELLAGAMBI
UNIAMO – Rare Diseases Italy
Italy

MR ALAIN CORNET
Lupus Belgium
Belgium

MS DORICA DAN
Romanian Prader Willi Association
Romania

MR JOHAN DE GRAAF
Dutch Pituitary Foundation
Netherlands

MR ALEXANDRE MEJAT
AFM - Téléthon
France

MS TATIANA KULESHA
Rare Diseases of Ukraine
Ukraine

MS GESKE WEHR
Selbsthilfe Ichthyose eV
Germany

MS KIRSTEN JOHNSON
The Fragile X Society
UK

MS ANNA SPINOU
Hellenic Cystic Fibrosis Association
Greece

MR DANIEL DE VICENTE
FEDER
Spain

MS REBECCA TVEDT SKARBERG
Osteogenesis Imperfecta Federation Europe (OIFE)
Norway

MR GRAHAM SLATER
Esophageal Atresia Global support groups
UK

MR TOMASZ GRYBEK
Foundation of Borys the Hero
Poland

BOARD OF OFFICERS

MAY 2024 - MAY 2025

PRESIDENT

MS AVRIL DALY
Retina International
Ireland

VICE-PRESIDENT

MS DORICA DAN
Romanian Prader Willi Association
Romania

MS SIMONA BELLAGAMBI
UNIAMO – Rare Diseases Italy
Italy

GENERAL SECRETARY

MS GESKE WEHR
Selbsthilfe Ichthyose eV
Germany

DEPUTY GENERAL SECRETARY

MS ANNA ARELLANESOVA
Rare Diseases Czech Republic
Czechia

TREASURER

MR ALAIN CORNET
Lupus Belgium
Belgium

EURORDIS MEMBERS 2024

ALBANIA

Shoqata e Semundjeve te Rralla / Rare Disease Association Albania	http://rda-al.com/	Associate Member

ALGERIA

Association Elamani pour venir en aide aux Malades souffrant de l'Anémie Héréditaire		Associate Member

ANDORRA

Associació de Malalties Minoritàries d'Andorra	https://amma.ad/	Full Member

ARGENTINA

Alianza Argentina de Pacientes	http://alianzapacientes.org/	Associate Member
Federación Argentina de Enfermedades Poco Frecuentes	http://fadepof.org.ar/	Associate Member

ARMENIA

Doctors And Children Health Care	http://www.rambler.ru	Associate Member
National Center for Rare Disease of Armenia	http://www.cmg.am	Associate Member

AUSTRALIA

Cystic Fibrosis Australia	http://www.cysticfibrosis.org.au	Associate Member
Genetic Alliance Australia	http://www.geneticalliance.org.au	Associate Member
Genetic Support Network of Victoria	https://www.gsnv.org.au/	Associate Member
Muscular Dystrophy WA	https://www.mdwa.org.au/	Associate Member
PURA Foundation Australia	https://www.purafoundation.au/	Associate Member
Rare Voices Australia	http://www.rarevoices.org.au	Associate Member
Save our Sons	https://www.saveoursons.org.au/	Associate Member

AUSTRIA

Angelman Verein Österreich	http://www.angelman.at	Full Member
Debra International	https://www.debra-international.org	Full Member
Hand in Hand Für Tay-Sachs & Palliativkinder	http://www.tay-sachs.net	Associate Member
ICA-Österreich	http://www.ica-austria.at	Full Member
Lasst uns MDS heilen - DupMECP2	https://dupmecp2.eu/	Full Member
NF Kinder- Hilfe für Neurofibromatose- PatientInnen und Angehörige, Österreich	http://www.nfkinder.at	Full Member
NF Patients United	http://www.nf-patients.eu	Associate Member
PH Austria - Initiative Lungenhochdruck	http://www.phaustria.org	Full Member
PHA Europe	http://www.phaeurope.org	Associate Member
Pro Rare Austria, Allianz für seltenen Erkrankungen	http://www.prorare-austria.org	Full Member

Smith-Magenis-Syndrom Österreich	https://www.smith-magenis.at/	Full Member
Usher Deafblind Forum Austria	http://www.usher-taubblind.at	Associate Member

BELARUS

Belarussian Organization of Patients with MPS and other Rare Genetic Disorders	http://www.mpssociety.by	Full Member

BELGIUM

22Q11 Europe	https://22q11europe.org/	Associate Member
Alpha-1 Europe Alliance asbl	https://alpha1europe.org/	Associate Member
Alpha-1 Plus Asbl	http://www.alpha1plus.be/	Associate Member
ALS Liga België	http://www.alsliga.be	Full Member
Amyloïdose Vereniging België	https://www.amybel.be/	Associate Member
Association Belge du Syndrome de Marfan Asbl	https://www.marfan.be/	Full Member
Association des Patients Sclérodermiques de Belgique	https://www.sclerodermie.be/	Full Member
Association Lupus Erythematosus	http://www.lupus-belgium.org	Full Member
Association pour l'Information et la Recherche sur les maladies rénales génétiques	http://www.airg-belgique.org	Associate Member
Belgische Organisatie Voor Kinderen En Volwassenen Met Een Stofwisselingsziekte	http://www.boks.be	Full Member
Belgische Vereniging voor Longfibrose VZW	http://www.longfibrose.org	Associate Member
BE-TSC VZW	http://www.betsc.be	Full Member
CF Europe	http://www.cf-europe.eu	Full Member
Children's Tumor Foundation Europe	https://www.ctfeurope.org/	Associate Member
Contactgroep Myeloom en Waldenström Patiënten	http://www.cmp-vlaanderen.be	Full Member
CURE KARS - Laia Foundation	https://www.curekars.org/	Associate Member
Debra Belgium	http://www.debra-belgium.org	Full Member
Euro Ataxia - European Federation of hereditary Ataxias	http://www.euroataxia.org	Full Member
European Chromosome 11 Network	https://chromosome11.org/en/home/	Full Member
European CMT Federation	http://www.ecmtf.org	Associate Member
European Federation of Williams Syndrome	http://www.eurowilliams.org	Full Member
European Haemophilia Consortium	http://www.ehc.eu	Full Member
European Liver Patients Association	https://elpa.eu/	Associate Member
European Myasthenia Gravis Association	https://www.eumga.eu/	Associate Member
European Network of Gynaecological Cancer Advocacy Groups	https://engage.esgo.org/	Associate Member
European Polio Union	http://www.europeanpolio.eu	Full Member
European Pulmonary Fibrosis Federation	http://www.eu-pff.org	Associate Member
Eye Hope Foundation	http://www.eyehopefoundation.org	Associate Member
Fabry International Network	https://www.fabrynetwork.org/	Associate Member
Familial Adenomatous Polyposis Association	http://www.belgianfapa.be/fr	Associate Member
Federation of European Scleroderma Associations	https://fesca-scleroderma.eu/	Associate Member
FEDERG - Federation of European Associations of Patients affected by Renal Diseases	http://federg.org	Full Member
Fragile X International	https://www.fraxi.org	Full Member
HTAP Belgique asbl	http://test.ph-vzw.be/fr_FR/	Full Member

Ichthyose Belgique - Ichthyosis België	http://www.ichthyosis.be	Full Member
International Federation for Spina Bifida and Hydrocephalus	http://www.ifglobal.org	Full Member
Liver Patients International	https://liverpatientsinternational.org/	Associate Member
Lupus Europe	http://www.lupus-europe.org	Associate Member
Mijnlever Patiëntenvereniging Zeldzame Leverziekten vzw	https://www.mijnlever.be/	Full Member
Myeloma Patients Europe	http://www.mpeurope.org	Associate Member
OIFE - Osteogenesis Imperfecta Federation Europe	http://www.oife.org	Full Member
RaDiOrg - Rare Diseases Belgium asbl/vzw	http://www.radiorg.be	Full Member
Rare Disorders Belgium	http://www.rd-b.be	Associate Member
Relais 22 Asbl	http://www.relais22.be	Full Member
Siop Europe - European Society for Paediatric Oncology	http://www.siope.eu	Associate Member
Spierziekten Vlaanderen VZW	https://spierziektenvlaanderen.be	Full Member
Steunpunt Kinderepilepsie vzw		Full Member
VASCAPA Vascular Anomaly Patient Association	http://www.vascapa.org	Full Member
Vlaams Patiëntenplatform vzw	https://vlaamspatientenplatform.be/nl	Associate Member
Vlaamse Vereniging voor erfelijke Bindweefselaandoeningen	http://bindweefsel.be	Full Member
vzw GEN	https://www.vzw-gen.be/	Associate Member
Zebrapad VZW	https://www.zebrapadvzw.be	Full Member

BENIN

Albinos Sans Frontières		Associate Member

BOSNIA AND HERZEGOVINA

Alliance for rare diseases of Republic of Srpska, Bosnia and Herzegovina	https://savezzarijetke.org/	Full Member
Association of Rare Disease Patients Bosnia and Herzegovina	https://www.facebook.com/udruzenjeoboljelihodrijetkihbolesti	Full Member

BRAZIL

Associação Brasileira de Amiloidose Hereditária	http://www.abpar.org.br/	Associate Member
Associacao Brasileira de Enfermedades Raras	http://feberraras.wixsite.com/feber-raras	Associate Member
Instituto Vidas Raras	http://www.vidasraras.org.br	Associate Member

BULGARIA

Association of Tarlov Cyst patients in Bulgaria	https://tarlov-bg.eu/	Full Member
Association of the patients with respiratory failure and lung transplantation	https://xn--80acal2ahwkf1m.bg/	Associate Member
Bulgaria society of patients with pulmonary hypertension	http://www.bspph.net	Full Member
Bulgarian Association of Moschcowitz Disease (TTP)	https://ttp-bg.eu/	Full Member
Bulgarian Association Wilson Disease	https://wilsonbg.org	Full Member
Bulgarian Cystic Fibrosis Association	https://lifewithcf.org/	Full Member
Bulgarian Huntington Association	http://huntington.bg	Associate Member
Bulgarian Lymphedema Association	https://lymphoedema-bg.org	Full Member
Bulgarian National Alliance of People with Rare Diseases	https://rare-bg.com/	Associate Member

FAST Bulgaria		Associate Member
Nataliya Foundation	https://www.nataliyafoundation.org/	Associate Member
National Association For Child Support Congenital Hypothyroidism		Associate Member
National Association of Patients With Growth Hormone Deficiency	https://www.facebook.com/Национална-Асоциация-на-пациенти те-с-дефицит-на-растежния-хормон-1543573779219269/?fref=hovercard	Full Member
National Association of Patients with Mitochondrial Diseases in Bulgaria	http://www.mitobg.com/	Full Member
National Gaucher Organization	https://gaucher-bg.org/	Associate Member
National Syringomyelia Association		Full Member
PHA Bulgaria		Full Member
Pituitary Association of Bulgaria	https://www.pituitary-bg.com/	Full Member
Rare Diseases Bulgaria	https://rare.bg/	Full Member
Retina Bulgaria	https://retinabulgaria.bg/	Full Member
Together Fighting Sarcoma	https://sarcoma.bg/	Associate Member

BURKINA FASO

Fondation Internationale Tierno et Mariam	http://www.fitima.org	Associate Member

CANADA

Canadian Organization For Rare Disorders	http://www.raredisorders.ca	Associate Member
International Alliance of Dermatology Patient Organizations	https://globalskin.org/	Associate Member
Lymphoma Coalition	https://lymphomacoalition.org/	Associate Member
PVNH Support & Awareness	http://www.pvnhsupport.com	Associate Member

CHINA

Chinese Organization for Rare Disorders	http://www.hanjianbing.org	Associate Member
Illness Challenge Foundation	http://www.chinaicf.org/	Associate Member

COLOMBIA

Asociación Colombiana de Pacientes con Enfermedades de Depósito Lisosomal	http://www.acopel.org	Associate Member
Foundation Diana Garcia de Olarte for PID	http://www.fundacion-fip.org	Associate Member

CROATIA

Croatian Rare Liver Foundation	https://www.rijetkebolestijetre.hr/about/	Associate Member
Debra Croatia	http://www.debra-croatia.com	Full Member
Dravet sindrome Croatia	http://dravet-sindrom-hrvatska.hr	Associate Member
Rare Diseases Croatia	https://rijetke-bolesti.com/	Full Member

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CYPRUS

Cyprus Alliance for Rare Disorders	http://raredisorderscyprus.com/	Full Member
Cyprus Association of Inherited Metabolic Diseases 'Aspida Zois'	http://www.aspidazois.com	Full Member
Pancyprian Association For Rare Genetic Diseases «Unique Smiles»	https://monadikaxamogela.org/	Full Member
Thalassaemia International Federation	http://www.thalassaemia.org.cy	Full Member
The Association of People with Primary Immunodeficiency and Friends		Full Member

CZECH REPUBLIC

AIPO z.s.		Associate Member
ALSA z.s.	https://www.zsalsa.cz/	Associate Member
Association of Atypical Parkinsonian Syndromes		Associate Member
HAE Junior	https://haejunior.cz/	Associate Member
Klub Nemocnych Cystickou Fibrozou	http://www.cfklub.cz	Full Member
Meta, Association of Patients with Lysosomal Storage Diseases	http://www.sdruzenimeta.cz	Full Member
Narodni Sdruzeni Pku A Jinych Dmp (Czech Pku Association)	https://www.nspku.cz/	Full Member
Rare Diseases Czech Republic (Ceska Ascociace Pro Vzacna Onemocneni)	http://www.vzacna-onemocneni.cz	Full Member
Spolecnost pro pomoc pri Huntingtonove chorobe, z.s./ Czech Huntington Association	http://www.huntington.cz	Associate Member

DENMARK

22Q11 Danmark	http://www.22q11.dk	Full Member
Blæreekstrofiforeningens	http://www.lfmb.dk	Full Member
CCHS Danmark	http://cchsdanmark.wordpress.com/	Associate Member
Danish Apert Syndrome Association /Danmarks Apertforening	http://www.apertforening.dk	Full Member
Danmarks Bloderforening / Danish Haemophilia Society	http://www.bloderforeningen.dk	Full Member
Ehlers-Danlos Foreningen I Danmark	http://www.ehlers-danlos.dk	Full Member
Foreningen for Ataksi / HSP	http://www.sca-hsp.dk	Full Member
HAE Scandinavia	https://haescan.org/	Full Member
Ichtyosis Association in Denmark	http://www.iktyosis.dk	Full Member
Ketotic Hypoglycemia International	http://ketotichypoglycemia.org/	Full Member
MCADD-Foreningen	http://www.mcadd.dk	Full Member
Möbius Forening Danmark / Moebius Syndrome Association Denmark	http://www.moebiussyndrom.dk	Full Member
Porfyriforeningen Danmark - Porphyria Association Denmark	https://porfyriforeningen.dk/	Full Member
Rare Diseases Denmark (Sjaeldne Diagnoser)	http://www.sjaeldnediagnoser.dk/	Full Member
The Danish Osteogenesis Imperfecta Society	http://www.dfoi.dk	Full Member
Wilson Patientforeningen	http://www.wilsons.dk	Full Member
XLH, arvelig rakitis (Hereditary Rickets Patient Association)	https://xlh-patientforeningen.dk/	Associate Member

ESTONIA

Estonian Prader Willi Syndrome Association	http://www.pws.ee	Full Member

FINLAND

Association of Cancer Patients in Finland (Suomen Syopapotilaat Ry)	http://www.syopapotilaat.fi	Full Member
Finnish Allergy, Skin And Asthma Federation	http://www.allergia.fi	Full Member
Finnish Association for Ultra-Rare Diseases	http://www.ultraharvinaiset.fi	Full Member
Finnish Association of People with Physical Disabilities	http://www.invalidiliitto.fi	Full Member
Finnish Neuro Society / Neuroliitto ry	https://www.neuroliitto.fi/	Full Member
Harso-Rare Disease Alliance Finland	https://www.harso.fi/en/home/	Full Member
Harvinaiset-Verkosto - Finnish Network For Rare Diseases	http://www.harvinaiset.fi	Associate Member
Inclusion Finland KVTL, Norio Centre of Rare Diseases	https://www.tukiliitto.fi/harvinaiskeskusnorio/	Full Member
SMA Finland ry	https://smafinland.fi/	Full Member
Suomen Akustikusneurinoomayhdistys ry/ Finnish Acoustic Neuroma Association	https://www.akustikusneurinoomayhdistys.com/	Full Member
Suomen Vaskuliittiyhdistys Ry / Finnish Vasculitis Association	https://www.vaskuliittiyhdistys.fi/	Full Member

FRANCE

ACO2 Gene Association	https://aco2gene.wixsite.com/asso	Associate Member
afa Crohn RCH France	http://www.afa.asso.fr	Associate Member
AFMKT-France	http://www.associationkystedetarlov.com	Full Member
AFM-Téléthon	http://www.afm-telethon.com/	Full Member
Agir pour la Malformation Lymphatique en Alliance	https://www.asso-amla.org/fr/	Full Member
Alliance Maladies Rares	https://alliance-maladies-rares.org/	Full Member
Alliance Syndrome de Dravet	http://www.dravet.fr	Full Member
Amis de ADNP France	https://www.alliance-maladies-rares.org/association/amis-de-adnp-france/	Associate Member
Amyloidosis Alliance the Voice of Patients	https://www.amyloidosisalliance.org/	Associate Member
ASL HSP-France	https://www.asl-hsp-france.org/	Full Member
Ass. Internationale De Dystrophie Neuro Axonale Infantile	http://www.nbiaalliance.org	Full Member
Assedea	http://www.assedea.fr	Full Member
Association ISIS	http://www.isis-asso.com	Full Member
Association «Turner et Vous»	http://turneretvous.org	Full Member
Association AMS-ARAMISE	http://www.ams-aramise.fr	Full Member
Association Anémies Dyserythropoïétiques Congénitales	https://filiere-mcgre.fr/le-parcours-patients/associations-de-malades/	Full Member
Association Bernard Pépin pour la Maladie de Wilson	https://www.abpmaladiewilson.fr/	Full Member
Association BPAN France	https://bpanfrance.fr/	Full Member
Association Contre Les Maladies Mitochondriales	http://www.association-ammi.org	Full Member
Association Craniopharyngiome Solidarité	http://www.cranio.fr	Full Member
Association des Groupes Amitié Turner	https://www.agat-turner.org/	Full Member
Association des Malades du Syndrome de McCune-Albright	http://www.assymcal.org	Full Member
Association des Malades Souffrant d'Angio Oedème par déficit en C1 Inhibiteur	http://www.amsao.fr	Full Member
Association des Pancréatites Chroniques Héréditaires	http://www.association-apch.org	Full Member
Association des Patients de la maladie de Fabry	http://www.apmf-fabry.org	Associate Member

Association des personnes concernées par le Tremblement Essentiel	http://www.aptes.org	Associate Member
Association du Naevus Géant Congénital	http://naevus.fr	Full Member
Association Enfants CASK France	https://www.aecf-france.fr/	Associate Member
Association Française contre l'Amylose	https://amylose.asso.fr/	Full Member
Association Française de Gilles de la Tourette	https://www.france-tourette.org/	Full Member
Association Française de La Maladie de Fanconi	http://www.fanconi.com	Full Member
Association Française de l'Ataxie de Friedreich	http://www.afaf.asso.fr/	Full Member
Association Française de l'Ostéogenèse Imparfaite	https://www.aoi.asso.fr/	Full Member
Association Française de Narcolepsie-Cataplexie et Hypersomnie	http://www.anc-narcolepsie.com	Full Member
Association Française des Dysplasies Ectodermiques	http://www.afde.net	Full Member
Association Française des Hémophiles	http://www.afh.asso.fr	Full Member
Association Française des Malades atteints de Porphyries	http://www.porphyries-patients.org	Associate Member
Association Française des Maladies Héréditaires du Rythme Cardiaque	http://www.afmhrc.org	Full Member
Association Française des Syndromes d'Ehlers-Danlos	http://www.afsed.com	Full Member
Association Française du Gougerot-Sjögren	http://www.afgs-syndromes-secs.org	Full Member
Association Française du Lupus et autres Maladies Auto-Immunes	http://www.lupusplus.com	Full Member
Association Française du Syndrome de Cornelia de Lange	http://afscdl.fr	Full Member
Association française du Syndrome de Guillain-Barré	https://syndrome-guillain-barre.fr	Full Member
Association Française du Syndrome de Klippel-Feil	https://afskf.fr/	Full Member
Association Francaise du Syndrome de Lowe	http://www.syndrome-lowe.org	Full Member
Association Française du Syndrome de Rett	https://afsr.fr/	Full Member
Association Française du Syndrome d'Ondine	https://afsondine.org/	Full Member
Association Française du Syndrome Phelan-Mcdermid	http://22q13.fr	Full Member
Association Française Lesch-Nyhan Action	http://www.lesch-nyhan-action.org	Full Member
Association Francaise Sturge Weber «Vanille-Fraise»	http://www.vanille-fraise.org	Associate Member
Association France Vascularites	https://www.association-vascularites.org/	Full Member
Association Francophone contre la Polychondrite Chronique Atrophiante	http://www.afpca.fr	Associate Member
Association Francophone de la Maladie de Blackfan Diamond	http://afmbd.org/	Full Member
Association Francophone des Glycogénoses	http://www.glycogenoses.org	Full Member
Association Francophone du Syndrome d'Angelman	http://www.angelman-afsa.org	Full Member
Association Gitelbart	https://gitelbart.fr	Full Member
Association Grandir	http://www.grandir.asso.fr	Full Member
Association Guerrier Mitochondrial	https://associationguerrie.wixsite.com/agmito	Associate Member
Association Histiocytose France	http://www.histiocytose.org	Full Member
Association Hypoparathyroidisme France	http://www.hypopara.fr	Full Member
Association Ichtyose France	http://www.ichtyose.fr	Full Member
Association Internationale Maladies Kystes Tarlov	http://www.aimktarlov.org	Associate Member
Association KCNB1 France	https://kcnb1-france.org/	Full Member
Association Kourir	http://www.kourir.org	Full Member
Association Maladies Foie Enfants	http://www.amfe.fr/	Full Member
Association Marfans	http://www.assomarfans.fr	Full Member
Association Microphtalmie France	http://asso-microphtalmie.org	Full Member

Association Naevus 2000	https://www.naevus2000.com/	Full Member
Association Nationale des Cardiaques Congénitaux	http://www.ancc.asso.fr	Full Member
Association Neurofibromatoses & Recklinghausen	http://www.anrfrance.fr	Full Member
Association Noonan	http://www.assonoonan.fr/	Full Member
Association Ollier Maffucci Europe	http://www.olliermaffucci-asso.fr/	Full Member
Association Pemphigus – Pemphigoïdes France	http://www.pemphigus.asso.fr	Full Member
Association Polyposes Familiales	http://www.polyposes-familiales.fr	Full Member
Association pour aider et informer les Syringomyéliques Européens Réunis	https://www.apaiser.org/	Full Member
Association pour la lutte contre l'Alcaptonurie	http://www.alcap.fr	Full Member
Association pour la Lutte contre les maladies Inflammatoires du Foie et des voies biliaires	http://www.albi-france.org	Full Member
Association pour la recherche sur la Sclérose Latérale Amyotrophique	https://www.arsla.org/	Associate Member
Association pour la Sensibilisation aux Maladies Rares, Orphelines et Auto-immunes à Saint-Pierre-et-Miquelon	https://maladiesraresspm.simdif.com/index.html	Full Member
Association pour l'aide aux personnes concernées par les Maladies Rares Muckle Wells Syndrome et CINCA	http://www.amws-caps.org/	Full Member
Association pour l'information et la prévention de la Drépanocytose	http://www.apipd.fr	Full Member
Association pour l'information et la recherche sur les Maladies Rénales Génétiques	http://www.airg-france.fr	Full Member
Association Sans Diagnostic et Unique	https://asdu.fr/	Associate Member
Association Sclérose Tubéreuse de Bourneville	http://www.astb.asso.fr	Full Member
Association SED1+	http://www.assosed1plus.com/	Full Member
Association SOS Desmoïde	http://www.sos-desmoide.asso.fr	Full Member
Association Spina Bifida et handicaps associés	http://www.spina-bifida.org	Associate Member
Association Surrénales	https://www.surrenales.com/	Full Member
Association Syndrome de Cowden	https://www.syndromedecowden.com/	Full Member
Association syndrome de Kleine-Levin	http://kls-france.org	Full Member
Association Syndrome de Moebius France	https://www.moebius-france.org/	Full Member
Association Syndrome PACS1 - Schuurs-Hoeijmakers	http://www.pacs1.org/	Associate Member
Association Tanguy Moya Moya	http://www.tanguy-moya-moya.org	Full Member
AVML Vivre Mieux le Lymphœdème	https://avml.fr/	Full Member
Charcot-Marie-Tooth France	http://www.cmt-france.org	Full Member
Cutis Laxa Internationale	http://www.cutislaxa.org	Full Member
Debra France	http://www.debra.fr	Full Member
Diabete Insipide France		Associate Member
Dravet Syndrome European Federation	https://dravet.eu/	Full Member
Dup15q France	https://www.dup15qfrance.fr	Full Member
Ectodermal Dysplasias International Network	https://edinetwork.org/	Associate Member
Enfants de la Lune Association pour le Xeroderma Pigmentosum	http://www.enfantsdelalune.org	Full Member
Ensemble Leucemie Lymphomes Espoir	https://www.ellye.fr/	Full Member
Espoir de Noisette	https://www.association-espoirdenoisette.org	Associate Member
Euro-Dyma	https://euro-dyma.eu/	Associate Member
European Federation for Hereditary Spastic Paraplegia	http://eurohsp.eu/	Full Member
European Federation Lesch-Nyhan Disease	http://www.LNDE.org	Associate Member

European Federation of Associations of Patients with Haemochromatosis	http://efaph.eu/	Full Member
European Patient Organisation for Dysimmune and Inflammatory Neuropathies	https://www.epodin.org/	Associate Member
EwenLife Rare Diseases	https://www.ewenlife.org/	Associate Member
FAST France	https://www.fastfrance.org/	Full Member
Fédération SOS Globi	https://sosglobi.fr/	Full Member
Fédération Williams France	http://www.williams-france.org	Full Member
Fitima Europe - Fondation International Tierno et Mariam	http://www.fitima.org	Full Member
FOP France	http://www.fopfrance.fr	Full Member
Fragile X France	http://www.xfra.org	Full Member
France Lymphangioleiomyomatose	http://www.francelam.org	Full Member
Fructos'Amis pour la Vie	http://www.fructosamispourlavie.org/	Full Member
Galactosémie France	https://www.galactosemie.fr/	Full Member
Génération 22	http://www.generation22.fr	Full Member
Genespoir: Association Française des Albinismes	http://www.genespoir.org	Full Member
Geniris	https://www.geniris.fr/	Full Member
HTaPFrance	http://www.htapfrance.com	Full Member
Hypophosphatasie Europe	http://www.hypophosphatasie.com	Full Member
Incontinentia Pigmenti France	http://incontinentia-pigmenti.fr/	Full Member
Inflam'Œil	https://www.inflamoeil.org/	Full Member
KCNQ2 France Développement	https://kcnq2francedeveloppement.fr/	Full Member
La Maison 8p	https://www.lamaison8p.fr	Associate Member
LAMA2 France, Contre les dystrophies musculaires par déficit en mérosine	https://www.lama2.fr/	Full Member
Les Amis de Remi	http://www.lesamideremi.fr	Full Member
Les Petits Bourdons	http://www.lespetitsbourdons.org	Associate Member
Let's Cure ACC	https://letscureacc.com/	Associate Member
LHF Espoir	https://lhfespoir.org/	Full Member
Ligue Nationale Contre Le Cancer	http://www.ligue-cancer.net	Full Member
Lupus France	http://www.lupusfrance.com/	Full Member
MED13L Syndrome association	http://www.med13lsyndrome.eu/	Associate Member
MNT Mon Poumon Mon Air	https://www.mntmonpoumonmonair.org	Full Member
Mosaïques - Association des «X Fragile»	http://www.xfragile.org	Full Member
Neuro IFF France et Maladies à Prions	https://neuroifffrance.my.canva.site/#accueil	Associate Member
Ouvrir Les Yeux	http://www.ouvrirlesyeux.org	Full Member
Petit Coeur de Beurre	https://www.petitcoeurdebeurre.fr	Full Member
Prader Willi France	https://www.prader-willi.fr/	Full Member
Retina France	http://www.retina.fr	Full Member
Sanfilippo Sud	https://www.sanfilippo-sud.com/	Full Member
SDTB, notre bataille	https://www.infos-sdtb.fr/fr/	Full Member
Solidarité Handicap autour des Maladies Rares	http://www.solhand-maladiesrares.org	Full Member
Tintamarre	https://www.asso-tintamarre.org/	Full Member
Union Nationale des Associations Parents et Amis Personnes Handicapées Mentales	https://www.unapei.org/	Associate Member
Union Nationale des Syndromes d'Ehlers-Danlos	http://www.unsed.org	Full Member

Vaincre Dunnigan	https://vaincre-dunnigan.org/	Associate Member
Vaincre la Mucoviscidose	http://www.vaincrelamuco.org	Full Member
Vaincre La Papillomatose Respiratoire Récurrente	http://www.vaincreprr.fr	Full Member
Vaincre Les Maladies Lysosomales	http://www.vml-asso.org	Full Member
Valentin APAC - Association de Porteurs d'Anomalies Chromosomiques	http://www.valentin-apac.org	Full Member
VHL France	http://www.vhlfrance.org	Full Member
Vivre sans Thyroide	https://www.forum-thyroide.net/	Full Member
White Sutton France	https://whitesuttonfrance.wixsite.com/	Associate Member
Xtraordinaire	http://www.xtraordinaire.org	Full Member

GEORGIA

Georgian Foundation for Genetic and Rare Diseases		Full Member
Georgian Rett syndrome and other rare diseases association	https://rettsyndrome.ge/	Associate Member
Primary Ciliary Dyskinesia Association Georgia		Associate Member
SCN2A Georgia		Associate Member

GERMANY

AHC-Deutschland e.V.	http://www.ahckids.de	Full Member
Allianz Chronischer Seltener Erkrankungen e.V.	https://www.achse-online.de/de/	Full Member
Angelman e.V.	http://www.angelman.de	Full Member
ARVC-Selbsthilfe e.V.	http://www.arvc-selbsthilfe.org	Full Member
BSHV für Kinder, Jugendliche und Erwachsene mit seltenen, chronischen Skeletterkrankungen e.V.	http://bshv-seltene-skelett-erkrankungen.com	Full Member
Bundesverband Angeborene Gefäßfehlbildungen e.V.	http://www.angiodysplasie.de	Full Member
Bundesverband für PFFD, FFU, Fibula- und Tibiadefekte e.V.	https://bupft.de/	Associate Member
Bundesverband Kleinwüchsige Menschen und ihre Familien e.V.	http://www.bkmf.de	Full Member
Bundesverband Niere e.V	https://www.bundesverband-niere.de/	Associate Member
Bundesverband Poliomyelitis e.V.	http://polio-selbsthilfe.de/willkommen	Full Member
Bundesverband Schilddrüsenkrebs - Ohne Schilddrüse Leben e.V.	https://www.sd-krebs.de/	Full Member
Bundesverein CDG-Syndrom e.V. / Glykokids	https://www.cdg-syndrom.de/ueber-uns.html	Full Member
Charge Syndrom e.V.	http://www.charge-syndrom.de	Full Member
Cystinose Selbsthilfe e.V.	http://www.leben-eben.com	Full Member
Deutsche Duchenne Stiftung	https://www.duchenne-deutschland.de/	Full Member
Deutsche Ehlers-Danlos Initiative e.V.	http://www.ehlers-danlos-initiative.de	Full Member
Deutsche GBS CIDP Selbsthilfe e.V.	http://gbs-selbsthilfe.org	Full Member
Deutsche Interessengemeinschaft PKU	http://www.dig-pku.de	Full Member
Deutsche Klinefelter-Syndrom Vereinigung e.V.	https://www.klinefelter.de	Full Member
Deutsche Sarkoidose Vereinigung e.V.	http://www.Sarkoidose.de	Full Member
Deutsche Syringomyelie und Chiari Malformation DSCM e.V.	http://www.deutsche-syringomyelie.de	Full Member
Deutsche Uveitis-Arbeitsgemeinschaft e.V.	http://www.duag.org	Full Member
Deutscher Verband für Kavernome (e.V.)	https://www.kavernome.de/	Associate Member
Deutschsprachige Selbsthilfegruppe für Alkaptonurie e.V.	http://www.dsaku.de	Full Member

dsai e.V. - Patientenorganisation für angeborene Immundefekte	http://www.dsai.de	Full Member
Dup15q e.V.	https://dup15q.de/	Full Member
EAT – Esophageal Atresia Global support groups	http://www.we-are-eat.org	Full Member
European Congenital Heart Disease Organisation	http://echdo.eu	Associate Member
European MEN Alliance	http://www.Emena.eu	Associate Member
European Network for Ichthyosis	https://ichthyosis.info/	Full Member
Faun Stiftung	http://faun-stiftung.de	Associate Member
FOP Germany (Förderverein für an Fibrodysplasia Ossificans Progressiva Erkrankte)	http://www.fop-ev.de	Full Member
Gaucher Gesellschaft Deutschland e.V.	https://www.ggd-ev.de/	Full Member
Gesellschaft für Mukopolysaccharidosen e.V.	http://www.mps-ev.de	Full Member
Glykogenose Deutschland e.V.	https://www.glykogenose.de/de/	Full Member
HAE Vereinigung e.V. (Hereditary Angioedema)	http://www.angiooedem.de	Full Member
Hand in Hand gegen Tay-Sachs und Sandhoff e.V.	http://tay-sachs-sandhoff.de/	Full Member
Hoffnungsbaum e.V. - Verein zur Förderung der Erforschung und Behandlung von NBIA-Erkrankungen	http://www.hoffnungsbaum.de	Full Member
HSP-Selbsthilfegruppe Deutschland e.V.	http://www.hsp-selbsthilfegruppe.de	Full Member
IEB e.V. DEBRA Deutschland	http://www.ieb-debra.de	Full Member
IMBS Alliance	https://www.imbs-alliance.org/	Associate Member
Interessengemeinschaft Fragiles-X e.V.	http://www.frax.de	Full Member
Interessengemeinschaft Hämophiler e.V.	https://www.igh.info/	Full Member
Kinder-Augen-Krebs-Stiftung	http://www.kinderaugenkrebsstiftung.de	Associate Member
Kindernetzwerk e.V.	http://www.kindernetzwerk.de	Associate Member
Kindness for Kids Foundation	http://www.kindness-for-kids.de	Associate Member
Leben mit Behcet in Deutschland	http://www.behcet-selbsthilfe.de	Associate Member
Leona e.V.	http://www.leona-ev.de	Full Member
Marfan Europe Network	http://www.marfan.eu	Full Member
Marfan Hilfe Deutschland e.V.	http://www.marfan.de	Associate Member
Morbus-Osler Selbsthilfe e.V	http://www.morbus-osler.de	Full Member
MPN-Netzwerk e.V.	http://www.Mpn-netzwerk.de	Full Member
Myelitis e.V.	https://www.myelitis.de/de/	Full Member
NCL-Gruppe Deutschland e.V.	http://www.ncl-deutschland.de	Full Member
Nephie e.V. - Selbsthilfe nephrotisches Syndrom	http://www.nephie.de	Full Member
Netzwerk Hypophysen- und Nebennierenerkrankungen e.V. - Network pituitary and adrenal disorders	http://www.glandula-online.de	Full Member
Patienten- und Selbsthilfeorganisation für Kinder und Erwachsene mit Kranker Speiseröhre	https://keks.org/	Full Member
Patientenverband Familiäre Amyloid Polyneuropathie	https://patientenverband-fap.de/	Associate Member
PCH-Familie e.V.	https://pch-familie.de/	Full Member
Peutz-Jeghers-Germany e.V.	http://peutz-jeghers.eu	Full Member
PKD Familiäre Zystenniere e. V.	http://www.pkdcure.de	Full Member
Pro Retina Deutschland e.V.	https://www.pro-retina.de/	Associate Member
Pulmonale Hypertonie e.V.	https://www.phev.de/	Full Member
PXE Selbsthilfegruppe Deutschland e.V.	https://pxe-shg.de/	Full Member
Sanfilippo Initiative e.V.	https://sanfilippoinitiative.org/en/home/	Associate Member

Sarcoidosis Europe	https://www.sarkoidose.de/epos/	Full Member
SCN2A Germany e.V.	https://www.scn2a.de/	Full Member
Selbsthilfe EPP e.V.	https://www.epp-deutschland.de	Full Member
Selbsthilfe Ichthyose e.V.	http://www.ichthyose.de	Full Member
Selbsthilfegruppe Ektodermale Dysplasie e.V.	http://www.ektodermale-dysplasie.de	Full Member
Selbsthilfegruppe für komplementbedingte Erkrankungen e.V.	https://www.ahus-selbsthilfe.de/	Full Member
Selbsthilfeorganisation für Menschen mit Anorektalfehlbildungen	http://www.soma-ev.de	Full Member
Sklerodermie Selbsthilfe e.V.	https://www.sklerodermie-sh.de/	Full Member
SMA Europe	https://www.sma-europe.eu/	Full Member
SSADH-Defizit e.V.	http://ssadh.wordpress.com	Associate Member
Syngap Elternhilfe e.V.	https://www.syngap.de	Full Member
Tom Wahlig Stiftung	https://www.hsp-info.de/	Full Member
Verein Ahc18+	https://www.ahc18plus.org/	Associate Member
Verein VHL (Von Hippel - Lindau) betroffener Familien e.V.	http://www.hippel-lindau.de	Full Member
Wir sind 22Q e.V.	https://www.wirsind22q.de/	Full Member
XP – Freu(n)de Mondscheinkinder	https://www.xerodermapigmentosum.de/	Full Member
ZNM - Zusammen Stark! e. V.	https://znm-zusammenstark.org/	Associate Member

GREECE

"95" - Rare Alliance Greece	http://www.rarealliance.gr	Full Member
AGORA – federation of associations for patients with rheumatic and musculoskeletal diseases of Southern Europe	https://www.agora-platform.eu/	Associate Member
Angelman syndrome Greece	https://angelman.gr/	Full Member
Association of Greek Friends for Paediatric Immunology Primary Immunodeficiencies «Harmony»	http://www.paed-anosia.gr	Associate Member
Child's Heart	http://www.kardiapaidiou.gr	Full Member
Hellenic Cystic Fibrosis Association	http://www.cysticfibrosis.gr	Full Member
Hellenic Friedreich's Ataxia Association	https://www.hefaa.org	Full Member
Hellenic League against Rheumatism	http://www.arthritis.org.gr	Associate Member
Hellenic Myasthenia Gravis Association	http://www.myasthenia.gr	Full Member
Immune Deficiency Association GALINOS	https://galinossilogos.wixsite.com/galinos	Associate Member
Karkinaki Awareness for childhood and adolescent Cancer	http://www.karkinaki.gr	Associate Member
KRIKOS ZOIS Society for Patients and Friends of Patients with Inherited Metabolic Disease	http://www.krikoszois.gr	Full Member
Muscular Dystrophy Association Hellas	http://mdahellas.gr	Full Member
Panhellenic Association of Patients & Friends with Neurofibromatosis «Life With NF»		Associate Member
Panhellenic Association of Patients with Lysosomal Disorders	http://greeklysosomal.gr	Full Member
Panhellenic Heart Diseases Organisation	http://kardiopatheia.gr	Full Member
Pespa (Greek Alliance for Rare Diseases)	http://www.pespa.gr	Associate Member
Prader Willi Syndrome Association Hellas		Full Member
Pulmonary Hypertension of Greece – Hellenic Pulmonary Hypertension	https://hellenicph.org/	Associate Member
Rare Diseases Greece	https://rarediseasesgreece.com/	Associate Member
To Mellon- Association of People with Genetic Disorders	http://www.tomellon.com.gr	Full Member

Together for Life		Full Member
VHLFA Alliance in Greece	https://www.vhlgr.org/	Associate Member

GUATEMALA

Asociación Nacional Guatemalteca para las Enfermedades de depósito Lisosomal	http://angel-lisosomal.org/	Associate Member
Procrece	https://www.facebook.com/asociacionprocreceguatemala/	Associate Member

HONG KONG, SAR OF CHINA

Joshua Hellmann Foundation for Orphan Disease	http://www.jhforphandisease.org/	Associate Member

HUNGARY

Hungarian Haemophilia Society - Magyar Hemofília Egyesület	http://www.mhe.hu	Full Member
Kéregtest rendellenesség AgCC Magyarországi Alapítványa / Disorder of the Corpus Callosum Hungary Fundation	https://www.agykeregtest.hu/	Associate Member
Magyaórszági Mitochondriális Betegek Alapítványa	http://www.nbia.hu	Full Member
Primer Immunhianyos Betegek Egyesülete	http://www.pibe.hu	Full Member
Rare Diseases Hungary - HUFERDIS	http://www.rirosz.hu	Full Member

ICELAND

AHC Federation of Europe	http://www.ahcfe.eu	Associate Member
Alternating Hemiplegia of Childhood Association of Iceland	http://www.ahc.is	Full Member
Einstök Börn - Support Group for Children with Rare Disorders	http://www.einstokborn.is	Full Member
Gudrun's Rett Syndrome Research Trust	http://rettenglar.yolasite.com	Associate Member
HD-Association of Iceland	https://www.huntington.is/	Full Member

INDIA

Indian Organization for Rare Diseases	https://www.rarediseases.in/	Associate Member
Organization For Rare Diseases India	https://ordindia.in/	Associate Member

IRAN (ISLAMIC REPUBLIC OF)

Rare Disease Foundation of Iran	http://radoir.org/fa/	Associate Member

IRELAND

22Q11 Ireland Support Group	http://www.22q11ireland.org	Full Member
Alpha One Foundation	http://www.alpha1.ie	Full Member
Barretstown Serious Fun	http://www.barretstown.org	Associate Member
Cystic Fibrosis Ireland	http://www.cfireland.ie	Associate Member
Cystinosis Ireland	http://cystinosis.ie	Full Member
Cystinosis Support Network Europe	https://cystinosis-europe.eu/	Associate Member
Debra Ireland	http://www.debraireland.org	Full Member
European Sickle Cell Federation	https://escfederation.eu/	Associate Member

Fighting Blindness	http://www.fightingblindness.ie	Full Member
Friedreich's Ataxia Research Alliance	https://faraireland.eu/	Full Member
Irish Cancer Society	http://www.cancer.ie	Associate Member
Irish MPS Society	http://www.mpssociety.ie/wordpress/	Full Member
Neurofibromatosis Association of Ireland	http://www.nfaireland.ie	Full Member
Pituitary Foundation Ireland	https://www.pituitaryireland.ie/	Full Member
Popsycle Foundation	https://www.facebook.com/Popsycle01/	Associate Member
Rare Diseases Ireland	http://rdi.ie/	Full Member
Rare Ireland	https://www.rareireland.ie/	Associate Member
Retina International	https://retina-international.org/	Full Member
Sickle Cell and Thalassaemie Ireland	http://www.sicklecellireland.ie	Full Member
Spina Bifida & Hydrocephalus Paediatric Advocacy Group	https://www.sbhpag.com/	Associate Member
The Cavan Tommy Hoey Trust	https://www.facebook.com/ the.cavan.tommy.hoey.trust/	Associate Member
The Irish Fragile X Society	http://fragilexireland.org	Full Member
TSC Ireland	https://tscireland.org/	Full Member
Usher syndrome Ireland	https://usherireland.org/	Associate Member
Vasculitis Ireland Awareness	http://www.vasculitis-ia.org	Full Member

ISRAEL

CdLS Israeli Foundation	https://www.cdlsisrael.org.il/	Associate Member
Coalition of Rare Diseases in Israel	https://www.rdisrael.org.il	Associate Member
GRIN Disorders Research Foundation	http://www.gringn.com/	Associate Member
Israel Adult Polyglucosan Body Disease		Associate Member
Little Steps Association	https://www.littlesteps.org.il/	Associate Member
NPO for promotion of health and cure of OPMD	https://opmd.health/en/home-page-en/	Associate Member

ITALY

A.C.A.R. Aps for MO/HME and Ollier/Maffucci syndrome	http://www.acar2006.org	Full Member
ABC Associazione Bambini Cri Du Chat	http://www.criduchat.it	Full Member
ACMT - Rete per la malattia di Charcot-Marie-Tooth OdV	http://www.acmt-rete.it	Full Member
Acondroplasia - Insieme Per Crescere - Onlus	http://www.acondroplasiaonlus.com	Full Member
AIBWS ODV	http://www.aibws.org	Full Member
AIMA-CHILD APS	https://www.aima-child.it/	Full Member
Angeli Noonan	http://www.angelinoonan.it	Full Member
Aniridia Italiana APS	http://www.aniridia.it/	Full Member
APS Bottega del Sorriso	https://www.apsbottegadelsorriso.it/	Full Member
Assi Gulliver - Associazione Sindrome di Sotos Italia	https://www.assigulliver.it/	Full Member
Associaizone Nazionale Pemfigo/Pemfigoide	https://www.pemfigo.org/	Full Member
Associazione Famiglie COL4A1-A2 APS	http://www.col4a1.it/	Full Member
Associazione Famiglie di Soggetti con Deficit dell'Ormone della Crescita ed altre Patologie	http://www.afadoc.it	Full Member
Associazione HHT Onilde Carini APS	https://www.hht.it/	Full Member

Associazione Italiana Adrenoleucodistrofia Onlus	https://www.adrenoleucodistrofia.it/	Associate Member
Associazione Italiana Cistite Interstiziale	http://www.aici-onlus.it	Full Member
Associazione Italiana Dei Cardiopatici Congeniti Adulti - Italian Guch Association	http://www.aicca.eu	Associate Member
Associazione Italiana Estrofia Vescicale-Epispadia ODV	http://www.estrofiavescicale.it	Full Member
Associazione Italiana Gaucher ODV	http://www.gaucheritalia.org	Full Member
Associazione Italiana Glicogenosi	http://www.aig-aig.it	Full Member
Associazione Italiana Laryngectomizzati	http://www.ailar.it	Associate Member
Associazione Italiana Leucodistrofie Unite	http://digilander.libero.it/ailu/	Full Member
Associazione Italiana Linfoistiocitosi Emofagocitica	https://www.aileonlus.org/	Associate Member
Associazione Italiana Malati di Alcaptonuria	http://www.aimaku.it	Full Member
Associazione Italiana Miastenia e Malattie Immunodegenerative - Amici del Besta Onlus	http://www.miastenia.it	Full Member
Associazione Italiana Miastenia Onlus	http://www.viverelamiastenia.it	Full Member
Associazione Italiana Morbo di Hirschsprung	http://amorhi.org	Associate Member
Associazione Italiana Mucopolisaccaridosi e Malattie Affini	http://www.aimps.it	Full Member
Associazione Italiana Niemann Pick Onlus	http://www.niemannpick.org	Full Member
Associazione Italiana Osteogenesi Imperfetta	https://www.asitoi.org/	Full Member
Associazione Italiana per la lotta alle PHTS	http://www.ptenitalia.org	Full Member
Associazione Italiana per le Malformazioni Anorettali	http://www.aimar.eu	Full Member
Associazione Italiana Porpora Immune Trombocitopenica aps	https://www.aipit.com/	Full Member
Associazione Italiana Rene Policistico Onlus	https://www.renepolicistico.it/	Full Member
Associazione Italiana Sindrome di Ehlers-Danlos	https://www.aised.it/	Full Member
Associazione Italiana Sindrome di Poland ODV	http://www.sindromedipoland.org	Full Member
Associazione Italiana Sindrome e malattia di Behçet	http://www.behcet.it	Full Member
Associazione Italiana Sindrome X Fragile	http://www.xfragile.net	Full Member
Associazione Italiana Siringomielia e Arnold Chiari	http://www.aismac.org	Full Member
Associazione Italiana Sostegno Malattie Metaboliche Ereditarie Onlus	http://www.aismme.org	Full Member
Associazione LAM Italia Onlus	http://www.lam-italia.org	Full Member
Associazione Ligure Thalassemici Onlus	http://www.althonlus.org	Full Member
Associazione Malattie Rare Dell'Alta Murgia Onlus	http://www.amaram.it	Associate Member
Associazione Nazionale Angioma Cavernoso Cerebrale	https://www.anaccaps.org/	Full Member
Associazione Nazionale Malattie Rare Dermatologiche Vascolari ODV	https://www.malattierare.gov.it/associazioni/dettaglio/981	Associate Member
Associazione Nazionale Persone con Malattie Reumatologiche	http://www.apmar.it	Full Member
Associazione p63 Sindrome E.E.C. International APS	http://www.sindrome-eec.it	Associate Member
Associazione per le Immunodeficienze Primitive ODV	http://www.aip-it.org	Full Member
Associazione per l'Informazione e lo Studio della Acondroplasia	https://aisac.it/	Full Member
Associazione Persone Williams Italia Onlus	http://www.apwitalia.org	Full Member
Associazione Poic e dintorni Onlus	https://www.poic-e-dintorni.org/	Full Member
Associazione S.P.R.IN.T.	https://www.associazionesprint.org/	Associate Member
Associazione Sclerosi Tuberosa	http://www.sclerosituberosa.org	Full Member
Associazione Sindrome di Alström / Italian Association for Alstrom Syndrome	https://www.alstrom.it/	Full Member
Associazione Sindrome Nefrosica Italia ODV	http://www.asnit.org	Full Member
Associazione Spina Bifida Italia	https://www.spinabifidaitalia.it/	Full Member

Associazione Studio Malattie Metaboliche Ereditarie Onlus	http://www.cometaasmme.org	Full Member
Associazione Tumori Torarici Rari Onlus	https://www.tumoritoracicirari.it/	Full Member
Associazione Veneta per la Lotta alla Talassemia	http://www.avlt.it	Full Member
CHAMP1 Foundation - Europe	https://www.champ1foundation.eu/	Associate Member
CIDP Italia Onlus	http://www.cidp.it	Full Member
Collagene Vi Italia Onlus - Col6	http://www.col6.it	Full Member
Costello.Cfc - Associazione Italiana Sindrome Di Costello - Cardiofaciocutanea - Rasopatie - Onlus	http://www.sindromedicostello.it	Full Member
Debra Italia Onlus	http://www.debraitalia.com/	Full Member
Dravet Italia Onlus	https://www.dravet.it/	Full Member
Federazione Italiana Prader-Willi	http://www.praderwilli.it	Full Member
Fondazione Alessandra Bisceglia ViVa Ale Onlus	http://www.fondazionevivaale.org	Associate Member
FOP Italia Onlus	http://www.fopitalia.it	Full Member
FSHD Italia APS	https://fshditalia.org/	Full Member
Gli Amici di Daniela	http://www.amicididaniela.it	Associate Member
Gli Equilibristi HIBM	https://www.gliequilibristi-hibm.org/	Full Member
Gruppo «Italia - Glioblastoma Multiforme - Cancro al Cervello»	https://www.facebook.com/groups/italia.glioblastoma.multiforme/	Associate Member
Gruppo Famiglie Dravet Associazione Onlus	http://www.sindromedidravet.org/	Full Member
Gruppo Familiari Beta-Sarcoglicanopatie	http://www.beta-sarcoglicanopathy.org/	Full Member
Gruppo Italiano per la lotta alla Sclerodermia Onlus	http://www.sclerodermia.net	Full Member
HHT Europe	http://www.hhteurope.org	Full Member
HHT Onlus	https://www.hhtonlus.org/	Full Member
Incontinentia Pigmenti Associazione Italiana Onlus	http://www.incontinentiapigmenti.it	Associate Member
La Strada per l'Arcobaleno	http://www.stradaperlarcobaleno.com	Full Member
Lega Italiana Ricerca Huntington	https://lirh.it/en	Full Member
Linfa Odv	https://www.linfaneurofibromatosi.com/	Full Member
LND Famiglie Italiane odv	http://www.lesch-nyhan.eu	Full Member
Mitocon - Insieme per lo Studio e la Cura delle Malattie Mitocondriali - Onlus	http://www.mitocon.it	Associate Member
NANA ETS	https://www.nanaets.org	Full Member
PANDAS Italia APS	https://pandasitalia.it/	Full Member
Parent Project aps	http://www.parentproject.it	Full Member
PKS Italia Aps	http://www.pksitalia.org	Full Member
Rari ma speciali ODV	https://www.malattiadikawasaki.it/	Full Member
Retina Italia odv	https://www.retinaitalia.org/	Full Member
Ring 14 International	http://www.ring14.org/eng/	Full Member
Sanfilippo Fighters odv	https://www.sanfilippofighters.org/en/homepage	Full Member
SCN2A ITALIA Famiglie in Rete APS	https://scn2a-italia.it/	Full Member
SCN8A Italia	https://www.scn8a.it/#	Full Member
SOD Italia - Associazione Septo Optic Dysplasia and Other Neuroendocrine Disorders	http://www.soditalia.it	Full Member
Un Filo per la Vita ANAD IICB	https://www.unfiloperlavita.it	Full Member
UNIAMO - Rare Diseases Italy	http://www.uniamo.org	Full Member
Unione Italiana Ittiosi	http://www.ittiosi.it	Full Member

Unione Italiana Lotta Alla Distrofia Musculare	http://www.uildm.org/	Full Member
Unione Trapiantati Polmone - Padova ODV	https://oltrelamalattia.it/	Full Member
United Onlus	https://www.unitedonlus.org/	Full Member
Uniti per la P.I.P.O.	https://www.unitiperlapipo.it/	Associate Member
World Federation of Incontinence Patients	http://www.wfip.org	Associate Member
XLPDR International Association	http://www.xlpdr.com/website/index.php	Associate Member

JAPAN

Japan Patient Association	https://nanbyo.jp/	Associate Member

KAZAKHSTAN

Association of Support to Patients with Orphan Diseases in the Republic of Kazakhstan	Associate Member
Patients with Cancer and Rare Diseases Support Association	Associate Member

KOSOVO

Rare Diseases Kosovo (Shoqata e Semundje te Rralla Kosove)	https://www.facebook.com/Shoqata-e-S%C3%ABmundjeve-t%C3%AB-Rralla-Kosov%C3%AB-2034022230042375/	Associate Member

LATVIA

Association of People with Special Needs «Motus Vita»	https://www.motusvita.lv/	Associate Member
Latvian Alliance for Rare Diseases	https://retasslimibas.lv/	Full Member
Rare Disease Association «Caladrius»	https://www.draugiem.lv/caladrius/	Associate Member

LEBANON

Lebanese Association for Neuromuscular Diseases	http://landforhope.org	Associate Member

LITHUANIA

Asociacija „Kraujas“	https://kraujas.lt/	Full Member
Rare Diseases Lithuania (Vaikų retų ligų asociacija)	http://retosligos.lt/	Associate Member

LUXEMBOURG

Alan - Maladies Rares Luxembourg	http://www.alan.lu	Full Member
EDS Lëtzebuerg a.s.b.l.	https://edsletzebuerg.lu/2/	Associate Member
Een Häerz Fir Kriibskrank Kanner Asbl	http://www.kriibskrankkanner.lu	Full Member
Rett Syndrome Europe	http://www.rettsyndrome.eu	Full Member
Syndrome de Marfan den-i.lu asbl	http://www.den-i.lu/	Associate Member

MALAYSIA

Malaysia Metabolic Society	https://mms.org.my/	Associate Member
Malaysian Rare Disorders Society	http://www.mrds.org.my	Associate Member

MALTA

National Alliance For Rare Diseases Support - Malta	http://www.rarediseasesmalta.com	Full Member

MEXICO

Asociacion de Gaucher de Mexico		Associate Member
Proyecto Pide Un Deseo Mexico Iap	http://www.pideundeseo.org	Associate Member
Red Sanfilippo	http://www.redsanfilippo.org	Associate Member

MONTENEGRO

National Organisation for Rare Diseases	http://www.rijetkebolesti.com	Associate Member

MOROCCO

Association Marocaine de la Fièvre Méditerranéenne Familiale et des autres Fièvres Récurrentes		Associate Member
Sun'Hop		Associate Member

NEPAL

GBS/CIDP Foundation Nepal		Associate Member
Muscular Dystrophy Organization Nepal	https://www.facebook.com/MuscularDystrophyOrganizationNepalPanautKavre/	Associate Member

NETHERLANDS

A Different Story	https://adifferentstory.nl/	Associate Member
AA & PNH Contact Group Foundation	http://www.aaenpnh.nl	Full Member
Acanthamoeba Keratitis Eye Foundation	https://akeyefoundation.com/	Associate Member
ALS patiëntenvereniging (ALS Patient Association)	https://www.alspatientenvereniging.nl/	Full Member
Amyloidose Nederland	http://amyloidose.nl	Full Member
Angelman Syndrome Alliance	https://angelmanalliance.org/	Full Member
Ataxie Vereniging Nederland	https://ataxie.nl/	Full Member
Bijniervereniging (Dutch Adrenal Patient Society)	http://www.nvacp.nl	Full Member
Childhood Cancer International	https://www.childhoodcancerinternational.org/	Full Member
Chordoma Foundation Europe	https://nl.chordomafoundation.org/	Full Member
CMTC-OVM	http://www.cmtc.nl	Full Member
Cornelia de Lange Syndrome World Federation	http://www.cdlsworld.org	Full Member
Cure ADOA Foundation	https://adoa.eu/	Full Member
European Cleft Organisation	http://europeancleft.org/	Associate Member
European Sarcoidosis Foundation	https://sarcoidosis.eu/	Associate Member
European Society for Phenylketonuria	http://www.espku.org	Associate Member
European VHL (Von Hippel-Lindau) Federation	https://www.vhl-europa.org/	Associate Member
Fabry Support & Informatie Groep Nederland	http://www.fabry.nl	Full Member
FH Europe Foundation	https://www.fheurope.org/	Associate Member

Fibrodysplasia Ossificans Progressiva Stichting Nederland	https://fopstichting.nl/	Full Member
FSHD Europe	http://fshd-europe.info	Full Member
Hevas	http://www.hevas.eu	Full Member
International Mito Patients	http://www.mitopatients.org	Full Member
International Painful Bladder Foundation	http://www.painful-bladder.org	Associate Member
International Porphyria Patient Network	https://porphyria.network/IPPN/	Associate Member
Interstitiële Cystitis Patientenvereniging	http://www.icpatienten.nl	Full Member
KAISZ - Children with a Autoinmuum or Autoinflammatory Disease	http://www.kaisz.nl	Full Member
LMNA Cardiac Foundation	https://www.lmnacardiac.org/	Associate Member
MSS (Marshall-Smith Syndrome) Research Foundation	https://marshallsmith.org/	Full Member
Naevus Global	https://naevusglobal.nevusnetwerk.nl/	Associate Member
National Association ReumaZorg Nederland	https://reumazorgnederland.nl/	Associate Member
Nationale Vereniging L.E. patiënten	http://www.nvle.org/	Full Member
Nederlands Netwerk voor Lymfoedeem en Lipoedeem	https://lymfoedeem.nl/	Associate Member
Nederlandse Hypofyse Stichting (Dutch Pituitary Foundation)	http://www.hypofyse.nl	Full Member
Nederlandse Leverpatienten Vereniging	http://www.leverpatientenvereniging.nl	Full Member
Nederlandse Phenylketonurie Vereniging / Dutch PKU Association	https://www.pkuvereniging.nl/	Full Member
Nederlandse Vereniging van Hemofilie-Patiënten/Netherlands Haemophilia Society	http://www.nvhp.nl	Full Member
Nephceurope	http://nephceurope.eu/nc/	Associate Member
Neurofibromatose Vereniging Nederland	http://www.neurofibromatose.nl	Full Member
Nevus Netwerk Nederland	https://nevusnetwerk.nl/	Full Member
Oscar Nederland	https://oscarnederland.nl/	Full Member
Patientenorganisatie Fibreuze Dysplasie	http://www.fibreuzedysplasie.eu	Full Member
Patiëntenvereniging voor Blaasextrofie Nederland	https://blaasextrofie.nl/	Full Member
Sarcoidose.Nl	http://www.sarcoidose.nl	Full Member
Spierziekten Nederland - Dutch Neuromuscular Patient Association	http://www.vsn.nl	Full Member
Stichting Hart4Onderzoek / Heart4Research	https://hart4onderzoek.nl/	Full Member
Stichting Histiocytose Nederland	https://www.histio.nl/	Full Member
Stichting Ijzersterk	http://www.stichtingijzersterk.nl	Associate Member
Stichting Kans voor PKAN Kinderen	https://kansvoorpkankinderen.nl	Full Member
Stichting Overdruksyndroom NL	https://sosnl.nl	Full Member
Stichting Pierre Robin Europe - Pierre Robin Europe Foundation	http://pierrerobineurope.com	Full Member
Stichting RPF Nederland / Dutch RPF Foundation	https://rpf.nl/	Full Member
Stichting Shwachman Diamond Syndrome Support Holland	http://www.shwachman.nl	Full Member
Stichting Spierkracht	https://stichtingspierkrac.wixsite.com/spierkracht	Associate Member
Stichting SynGAP Research Fund Europe	https://www.syngapresearchfund.org/#googtrans(en|en)	Full Member
Stichting TAPS Support/ TAPS Support Foundation	https://www.stichtingtapssupport.com/	Associate Member
Stichting Terre - Rett Syndrome Foundation	https://stichtingterre.nl/	Full Member
Stichting voor Afweerstoornissen	http://www.stichtingvooraf weerstoornissen.nl	Full Member
Stichting Zeldzame Bloedziekten	http://www.bloedziekten.nl	Associate Member
Thyroid Cancer Alliance	https://www.facebook.com/ThyroidCancerAlliance/	Full Member

Vasculitis Stichting	http://www.vasculitis.nl	Full Member
Vereniging van Ehlers Danlos Patienten	https://ehlers-danlos.nl/	Full Member
Vereniging voor Ichthyosis Netwerken	https://ichthyosisnetwerken.nl/	Full Member
Volwassenen, Kinderen En Stofwisselingsziekten	https://www.stofwisselingsziekten.nl/	Full Member
VSOP - Vereniging Samenwerkende Ouder En Patiëntenorganisaties	https://vsop.nl/	Full Member
World Alliance of Pituitary Organizations	http://www.wapo.org	Associate Member
World Duchenne Organization	https://www.worldduchenne.org/	Full Member

NEW ZEALAND

Rare Disorders NZ	http://www.nzord.org.nz	Associate Member

NORTH MACEDONIA

APRDD My rare world		Associate Member
Association for help and support of patients and their caregivers with Haematological Diseases	http://www.hema.org.mk	Associate Member
Association for persons with Spinal Muscular Atrophy - Stop SMA	https://www.facebook.com/STOP-SMA-Macedonia-%D0%A1%D0%A2%D0%9E%D0%9F-%D0%A1%D0%9C%D0%90-1916753535041997/	Associate Member
FEMINA M Skopje	http://www.feminam.org.mk/	Associate Member
Life With Challenges	http://challenges.mk/?p=2288&lang=en	Full Member
National Alliance For Rare Diseases of North Macedonia		Full Member
Save Liver Association of Patients with Liver Diseases	https://www.facebook.com/udrugaslap/	Full Member

NORWAY

Aniridia Europe	http://www.aniridia.eu	Full Member
European Huntington Association	http://eurohuntington.org/	Full Member
Frambu - Resource Centre For Rare Disorders	https://frambu.no/	Full Member
HHT-Osler foreningen, Norge	http://www.osler.no	Full Member
Hjernesvulstforeningen (Norwegian Brain Tumour Association)	https://hjernesvulst.no/	Full Member
Hypopara Norge	https://www.hypopara.no/	Full Member
International Huntington Association	https://huntington-disease.org/	Associate Member
MPS-Foreningen I Norge	http://mpsforeningen.no/	Associate Member
Norsk Forening For Ehlers-Danlos Syndrom	http://www.eds-foreningen.no	Full Member
Norsk Forening for Osteogenesis Imperfecta/ The Norwegian Osteogenesis Imperfecta Association	http://www.nfoi.no	Full Member
Norsk Forening For Tuberos Sklerose	http://www.nfts.no	Full Member
Norwegian Federation of Organisations of Disabled People (Funksjonshemmedes Fellesorganisasjon)	https://www.ffo.no/	Full Member
Norwegian organisation for Prader Willis syndrome	https://www.prader-willis.no/	Full Member

POLAND

3majmy sie razem - Let's get together	https://3majmysierazem.pl/	Associate Member
Debra Polska	http://www.debra-kd.pl/	Full Member

FAST Poland	https://www.cureangelman.pl/	Associate Member
Foundation for Leukemia Patients/Fundacji Na Rzecz Pomocy Chorym Na Bialaczki	https://fundacja.hematologiczna.org/	Associate Member
Foundation of Borys the Hero /Fundacja Bohatera Borysa	http://bohaterborys.pl/	Full Member
Fundacja Celując w Przyszłość / Aiming for the future Foundation	https://celujacwprzyszlosc.org/en	Associate Member
Fundacja Lelka /Lelek Foundation	https://fundacjalelka.pl/	Full Member
Fundacja Sanfilippo	http://sanfilippo.org.pl/en/home/	Associate Member
Fundacja SMA (SMA Foundation Poland)	http://www.fsma.pl	Full Member
Fundacja Umiec Pomagac (Foundation For RD MPS)	http://www.umiecpomagac.org	Full Member
Krajowe Forum Orphan/National Forum Orphan	http://rzadkiechoroby.org/	Full Member
Matio Fundacji Pomocy Rodzinom I Chorym Na Mukowiscydozę/MATIO Polish CF Foundation	http://www.mukowiscydoza.pl	Full Member
PACS2 Research Foundation	https://www.pacs2research.org/	Full Member
Polish PKU and RD Association «Ars Vivendi»	http://www.fenyloketonuria.org	Full Member
Polish Society of MPS and Related Diseases	https://chorobyrzadkie.pl/	Full Member
Polskie Stowarzyszenie Na Rzecz Osób Z AHC	http://www.stow.ahc-pl.org/	Associate Member
Rett Syndrome Poland - Ogólnopolskie Stowarzyszenie Pomocy Osobom Z Zespołem Retta	https://rettsyndrome.pl/	Full Member
The Dina Radziwillowa Child's Heart Foundation	https://www.sercedziecka.org.pl/	Full Member

PORTUGAL

andLINFA|Associação Nacional de Doentes Linfáticos / National Association of Sufferers of Lymphatic Disorders	https://andlinfa.pt/	Full Member
Associação Nacional De Displasias Ósseas	http://www.andoportugal.org	Associate Member
Associação Nacional para Divulgar e Orientar para Combater e Enfrentar a Tay Sachs e outras Gangliosidoses	http://doce.pt/wp/	Associate Member
Associacao Portuguesa CDG e Outras Doencas Metabolicas	https://www.facebook.com/SINDROMECDG/?locale=pt_BR	Associate Member
Associação Portuguesa De Charcot-Marie-Tooth	https://www.facebook.com/associacao.portuguesa.charcot.marie.tooth/	Full Member
Associação Portuguesa De Insuficientes Renais	http://www.apir.org.pt	Full Member
Associaçao Portuguesa de Leucemias e Linfomas	http://www.apll.org	Full Member
Associaçao Portuguesa De Neuromusculares	https://apn.pt/apn/	Full Member
Associação Portuguesa de Osteogénese Imperfeita	http://www.apoi.pt	Full Member
Associação Sanfilippo Portugal	http://www.sanfilippoportugal.com	Full Member
EVITA	http://www.evitacancro.org/	Associate Member
Fedra - Federaçao Portuguesa De Doenças Raras	http://www.fedra.pt	Associate Member
Liga Portuguesa contra as Doenças Reumáticas	http://www.lpcdr.org.pt	Full Member
Oncological and Gynaecological Movement	https://mogportugal.pt/	Associate Member
Raramente		Associate Member
Rarissimas - Associacao Nacional De Deficiencias Mentais E Raras	https://rarissimas.pt/	Full Member
RD-Portugal - União de Associações das Doenças Raras de Portugal	https://raras.pt/	Associate Member

PHOTO: LIGHT UP FOR RARE - AUSTRALIA

REPUBLIC OF MOLDOVA

AO SOS Autism	https://autismmoldova.md/	Associate Member
Copiii Ploii	https://www.facebook.com/AO-Copiii-Ploii-686485228086936/	Full Member

ROMANIA

Asociatia Copiilor Cu Boli Mitocondriale/ Association of Children with Mitochondrial Diseases	https://cure-echs1.com/	Associate Member
Asociatia Copilul Meu-Inima Mea	http://www.acmim.ro	Full Member
Asociatia Nationala Miastenia Gravis Romania / Romanian Myasthenia Gravis Association	http://www.miastenie.ro	Full Member
Asociatia Pentru Matei	https://www.pentrumatei.ro/	Full Member
Asociatia Persoanelor Cu Glicogenoza (APG Romania)	https://glicogenoza.ro/	Associate Member
Asociația Română De Cancere Rar/ Romanian Association for Rare Cancers	http://www.arcrareromania.ro	Associate Member
Asociatia Romana pentru Boli Neurologice Periferice/ Romanian Association of Perypheral Neurological Diseases	https://arbnp-cidp.ro/	Full Member
Asociatia Romana Spina Bifida si Hidrocefalie	https://www.facebook.com/ARSBH/	Associate Member
Asociatia Sindromul Coffin-Lowry/Coffin-Lowry Syndrome Association	http://www.coffin-lowry.ro/	Associate Member
Asociatia Smacare	http://www.amiotrofie-spinala.ro	Full Member
Asociatia Werdnig Hoffman Awh	https://www.facebook.com/werdnighoffman/	Full Member
Autoimmune Diseases Patients Association	http://www.apaa.ro	Associate Member
Charcot Marie Tooth Romania Association	http://www.asociatiacmt.ro	Associate Member
DMD Care	https://dmdcare.org	Full Member
Mastocytosis Support Association Romania	http://www.mastocitoza.ro	Associate Member
Neuro Move CMT Association	https://neuromovecmt.ro/	Associate Member
Romanian National Alliance for Rare Diseases	https://bolirareromania.ro/	Full Member
Asociatia Copiilor Cu Boli Mitocondriale/ Association of Children with Mitochondrial Diseases	https://cure-echs1.com/	Associate Member

RUSSIAN FEDERATION

Help to Cystic Fibrosis Patients		Full Member
Interregional Public Organisation For Gaucher Disease	https://www.gaucher.ru/	Full Member
Interregional Public Organisation of Patients with Fabry Disease «The Road To Life»	https://ufafabry.ru/	Full Member
Inter-Regional Support Centre for Patients with Aniridia « Iris»	https://aniridia.ru/	Associate Member
Look to see	https://looktosee.ru/	Full Member
MPS Russia	http://www.mps-russia.org	Full Member
National Association of Organization of Patient with Rare Diseases «Genetics»	https://nacgenetic.ru/	Associate Member
NGO «Fragile Children»	https://osteogenez.ru/	Full Member
Russian Association of Rare Diseases	https://www.rare-diseases.ru/	Full Member
Russian Patient Association	https://vspru.ru/en	Associate Member
Russian Rett Syndrome Asociation	https://radugaclinic.ru/	Associate Member
SPIPORZ Union of patients with rare diseases and rare disease patients organsiations	https://spiporz.ru/	Associate Member
The Association of Primary Immunodeficiency Patients	https://www.oppid.ru/	Full Member

RWANDA

Centre Alliance for Rare Diseases	https://centre-alliance.rw/	Associate Member

SERBIA

Child Rare Disease Support and Research Association Life	http://www.zivotorg.org	Associate Member
Citizens Association «Bromologos»	https://fenilketonurija.com/	Associate Member
DMD Serbia	http://dmdsrbija.rs/	Full Member
Lil' Brave One (Hrabrisa)	https://www.hrabrisa.rs/en	Full Member
Lymphoma Patients' Association	http://www.lipa.org.rs	Associate Member
National Organization For Rare Diseases of Serbia	http://www.norbs.rs	Full Member

SINGAPORE

Rare Disorders Society (Singapore)	https://rdss.org.sg/	Associate Member

SLOVAKIA

Debra SR	http://www.debra-slovakia.org	Full Member
Organisation of Muscular Dystrophy in the Slovak Republic/ Organizácia muskulárnych dystrofikov v SR	http://www.omdvsr.sk/	Full Member
Slovak Alliance of Rare Diseases	http://www.sazch.sk	Full Member
Slovak Cystic Fibrosis Association	https://www.cfasociacia.sk/	Full Member
Zdruzenie Ojedlinelych Genetickych Ochorenie	http://www.zogo.sk/	Associate Member

SLOVENIA

Association of Patients with Rare Eye Diseases Svetloba	https://drustvosvetloba.si/	Full Member
Debra Slovenia - Drustvo Debra Slovenija	http://www.debra-slovenia.si	Full Member
Drustvo bolnikov s krvnimi boleznimi Slovenije/ Association of Patients with Blood Diseases	http://www.drustvo-bkb.si	Full Member
EAMDA - European Alliance of Neuromuscular Disorders Associations	http://www.eamda.eu/	Full Member
European Foundation for SATB2-Associated Syndrome (SATB2 Europe)	https://www.satb2europe.org/	Associate Member
Fabry Patients Association Slovenia / Društvo Bolnikov S Fabryjevo Boleznijo Slovenije	http://www.sb-sg.si	Full Member
Foundation of Child Neurology	http://pednevro.pedkl.si/english/foundation/	Associate Member
IDefine Europe - Foundation for the Advanced Treatment of Rare Genetic Diseases	https://idefine-europe.org/	Associate Member
Vesele nogice (Happy Feet)	http://drustvo-veselenogice.si/	Associate Member
Viljem Julijan Association for Children with Rare Diseases	http://viljem-julijan.si/	Full Member
Zavod Bernardi Ventrella	https://www.zavodbv.org/	Associate Member

SOUTH AFRICA

Primary Immunodeficiency Network of South Africa	https://pinsa.org.za/	Associate Member
Rare Diseases South Africa NPC	https://www.rarediseases.co.za/	Associate Member

SPAIN

Acción y Cura Para Tay-Sachs	http://www.actays.org	Full Member
AHUCE - Asociación Nacional Huesos de Cristal	http://www.ahuce.org	Full Member
Alianza Española de Familias de von Hippel Lindau	http://www.alianzavhl.org	Full Member
AMAL Asociacion Madrilena de Afectados de Linfedema y Lipedema	https://www.amalmadrid.com/	Full Member
AMC - Artrogriposis Múltiple Congénita - España	https://artrogriposis.org/	Full Member
Asociació Catalana de las Neurofibromatosis	http://www.acnefi.com/castella/gamhome.htm	Full Member
Asociacion Albi España	http://www.asociacionalbi.com/	Full Member
Asociacion Andaluza de Fibrosis Quística	http://fqandalucia.org/	Full Member
Asociación Andaluza de pacientes con Síndrome de Tourette y Trastornos Asociados	http://www.tourette.es	Full Member
Asociación Atrofia del Nervio Optico de Leber	https://www.asanol.com/	Full Member
Asociación Chiari y Siringomielia del Principado de Asturias	http://www.chyspa.org	Full Member
Asociación Ciudadana de Afectados de Cistitis Intersticial	https://www.acaci.es/	Full Member
Asociación de Acondroplasia y otras Displasias Esqueléticas con Enanismo	https://adee.es/	Full Member
Asociación de Afectados CDKL5	https://aacdkl5.org	Full Member
Asociación de Afectados de Neurofibromatosis	http://www.neurofibromatosis.es	Full Member
Asociación de Afectados por Displasia Ectodérmica	https://displasiaectodermica.org/	Full Member
Asociacion De Enfermedades Raras D'genes	http://www.dgenes.es/	Full Member
Asociación de Epidermolisis Bullosa de España (Debra Spain)	http://www.pieldemariposa.es	Full Member
Asociación de Esclerodermia Castellon	https://www.esclerodermia.es/	Full Member
Asociación de Familiares y Afectados por Lipodistrofias	http://www.aelip.org	Full Member
Asociación de Hemoglobinuria Paroxística Nocturna	https://hpne.es/	Associate Member
Asociación de Nevus Gigante Congénito	http://asonevus.org/	Full Member
Asociación de pacientes ASMD España	https://www.asmd.es/	Full Member
Asociación de Sarcomas Grupo Asistencial	https://www.asarga.es/	Full Member
Asociación de Síndrome de Lamb-Shaffer España	https://www.lambShaffer.es	Associate Member
Asociación de Uveítis - AUVEA	https://www.asociacionauvea.es/	Full Member
Asociación Enfermedad de Kawasaki	https://asenkawa.org/	Full Member
Asociación Española Aniridia	http://www.aniridia.es	Full Member
Asociación Española de Amiloidosis	https://www.amilo.es/	Associate Member
Asociación Española de Angioedema Familiar	http://www.angioedema-aedaf.org	Full Member
Asociación Española de Enfermos de Glucogenosis	https://www.glucogenosis.org/	Full Member
Asociación Española de Enfermos de Pompe	http://www.asociaciondepompe.org	Associate Member
Asociación Española de Enfermos y Familiares de la Enfermedad de Gaucher España	http://www.aeefegaucher.es	Full Member
Asociación Española de Esclerodermia	https://esclerodermia.com	Full Member
Asociación Española de Familiares y Enfermos de Wilson	https://enfermedaddewilson.org/	Full Member
Asociación Española de Fibrodisplasia Osificante Progresiva	http://www.aefop-es.org	Full Member
Asociación Española de Fiebre Mediterranea Familiar	https://fmf.org.es/	Full Member
Asociación Española de Ictiosis	http://www.ictiosis.org	Full Member

Asociación Española de Mastocitosis y Enfermedades Relacionadas	http://www.mastocitosis.com	Full Member
Asociación Española de Pacs1	http://pacs1.es/	Associate Member
Asociación Española de paraparesia espástica familiar Strümpell-Lorrain	http://www.aepef.org	Full Member
Asociación Española de Porfiria	http://www.porfiria.org	Full Member
Asociación Española de Raquitismos y Osteomalacia Heredados	https://aeryoh.org/	Associate Member
Asociación Española de Síndrome De Poland	http://www.aesip.es	Full Member
Asociación Española de Sindrome Tatton Brown Rahmna		Full Member
Asociación Española de Síndromes Compresivos Vasculares	https://www.aescov.es/	Full Member
Asociación Española de Sjögren	http://www.aesjogren.org	Full Member
Asociación Espanola deficit de Lipasa Acida Lisosomal	http://www.aelald.org	Full Member
Asociación Española del sindrome CDG	http://aescdg.com/	Associate Member
Asociación Española del Síndrome de Schaaf-Yang	https://www.aesys.org/	Full Member
Asociación HHT España	http://www.asociacionhht.org	Full Member
Asociación KIF1A España	https://kif1a.es/	Full Member
Asociación Kleefstra España	https://www.kleefstra.es/	Associate Member
Asociación Madrileña de Pacientes con Sindrome de Gille de la Tourette y Trastornos Asociados	https://www.ampastta.com/	Full Member
Asociación Mi Princesa Rett	https://miprincesarett.es/	Full Member
Asociación Nacional Amigos De Arnold Chiari	http://www.arnoldchiari.es/	Full Member
Asociación Nacional de Afectados por Sindromes de Ehlers Danlos e Hiperlaxitud	http://ansedh.org/	Full Member
Asociacion Nacional de Dermatomiositis Juvenil	https://anadeju.org/	Full Member
Asociación Nacional de Hipertensión Pulmonar	https://hipertensionpulmonar.es/	Full Member
Asociación Nacional Síndrome de Apert y otras Craneosinostosis Sindrómicas	http://www.ansapert.org	Full Member
Asociación Retina Murcia	https://www.retinamurcia.org/	Full Member
Asociación Síndrome de Angelman	https://angelman-asa.org/	Full Member
Asociacion sindrome de la persona rigida-stiffperson-sps	https://asociacionsindrome personarigida.es/	Full Member
Asociación Stop Sanfilippo	https://www.stopsanfilippo.org/	Associate Member
Asociación Xeroderma Pigmentosum	https://xerodermapigmentosum.es/	Full Member
Associacio Antian		Associate Member
Associacio Catalana de la Deleccio 22Q	http://www.22q.cat	Full Member
Associació d'Apràxia Ocular i Malalties Associades	http://apraxiaocular.blogspot.com	Associate Member
Associación Catalana de Enfermedades Neuromusculares	http://www.asemcatalunya.com	Full Member
De Neu - Asociación De Enfermedades De Los Neurotransmisores	http://www.deneu.org	Full Member
Duchenne Parent Project España	http://www.duchenne-spain.org	Full Member
Enfermedades Raras Mas Visibles	https://www.masvisibles.com/	Associate Member
European Network For Rare And Congenital Anaemias	http://www.enerca.org	Associate Member
FEDER - Federación Española de Enfermedades Raras	http://www.enfermedades-raras.org	Full Member
Federació Catalana de Malalties Minoritàries	https://www.fecamm.org/portal1/m_index.asp?idioma=1	Full Member
Federacion de Asociaciones de Distrofias Hereditarias de Retina de España	http://www.retinosisfarpe.org	Full Member
Federación Española de Enfermedades Neuromusculares	http://www.asem-esp.org	Full Member
Federación Española de Familias de Cáncer Infantil	https://www.cancerinfantil.org	Full Member
Federación Española De Fibrosis Quistíca	http://www.fibrosis.org/	Full Member
Federación Española De Hemofilia	http://www.hemofilia.com	Full Member

Federación Española del Síndrome X Frágil	http://www.xfragil.org	Full Member
Fundación ALPE Acondroplasia	https://www.fundacionalpe.org	Full Member
Fundación Andrés Marcio, Niños Contra La Laminopatía	http://www.fundacionandresmarcio.org	Full Member
Fundación contra La Hipertensión Pulmonal	https://www.fchp.es/	Associate Member
Fundacion Libellas	https://fundacionlibellas.org	Associate Member
Fundación Mari Paz Jiménez Casado	https://www.fundacion maripazjimenez.org	Full Member
Fundación Niemann-Pick de España	http://www.fnp.es	Full Member
Fundacion Noelia, Collagen VI Deficiency Muscular Dystrophy	https://fundacionnoelia.org/	Full Member
Fundación Síndrome 5P Menos	http://www.fundacionsindrome5p.org	Full Member
Fundación Síndrome de Dravet - Dravet Syndrome Foundation	http://www.dravetfoundation.eu	Full Member
Fundacion Sindrome De West	http://www.sindromedewest.org	Full Member
Fundación Síndrome Wolf Hirschhorn 4P	https://www.fundacion4pmenos.com	Full Member
Hipertension Pulmonar España Organizacion de Pacientes	https://hipertension-pulmonar.com/	Associate Member
Instituto de Investigación y Desarrollo Social de Enfermedades Poco Frecuentes	http://www.pocofrecuentes.org	Associate Member
Menkes International Association	https://menkesinternational.com/	Full Member
MPS Lisosomales España	http://www.mpsesp.org/	Full Member
Pequeños Superheroes	https://pequenossuperheroes.org/	Associate Member
SAF España	https://www.antifosfolipido.es/	Full Member
SAMS - Asociacion para la lucha contra los Sindromes Arrítmicos Relacionados con la Muerte Súbita	https://www.samsasociacion.com/	Full Member
Sense Barreres de Petrer	https://sensebarreres.es/	Associate Member
SIMA Asociación de afectados Síndrome de Marfan	http://www.marfan.es/	Full Member

SWEDEN

Agrenska	http://www.agrenska.se	Full Member
Aorta Dissektion Föreningen Skandinavien	https://aortadissektion.com/en/	Full Member
DysNet	https://www.dysnet.org/	Full Member
European Cavernoma Alliance	https://sites.google.com/ cavernostangiomsverige.org/eca/home	Associate Member
Neuroförbundet / Swedish Association Of Persons With Neurological Disabilities	http://neuroforbundet.se	Associate Member
Prader Willi Syndrome Association in Sweden	https://prader-willi.se/	Full Member
Primär Immunbrist Organisationen	http://www.pio.nu	Full Member
Rare Diseases Sweden (Riksförbundet Sällsynta Diagnoser)	http://www.sallsyntadiagnoser.se	Full Member
Svenska Marfanföreningen - Swedish Marfan Asscіation	http://www.marfanforeningen.se/	Full Member
Svenska Ödemförbundet (Swedish Assocation of Chronic Oedema)	https://www.svenskaodemforbundet.se/	Associate Member
Swedish Cystic Fibrosis Association	https://www.rfcf.se/	Full Member
Swedish EDS Association (EDS Riksförbund)	https://ehlers-danlos.se/	Full Member
Swedish MPS Society (Svenska MPS Föreningen)	https://mpsforeningen.se/	Full Member
Thyroid Federation International	https://thyroid-fed.org/	Associate Member
Wilhelm Foundation	https://wilhelmfoundation.org/	Full Member

SWITZERLAND

AGO Alliance	https://argonautes.ngo	Associate Member
Association Enfance et Maladies Orphelines	http://www.aemo.ch	Associate Member
Association OrphanHealthcare, for Families with Rare Diseases	http://www.orphanhealthcare.org/	Associate Member
Blackswan Foundation	http://www.blackswanfoundation.ch	Associate Member
CML Advocates Network	http://www.cmladvocates.net	Associate Member
Esperare	http://www.esperare.org	Associate Member
FMF & AID Global Association	http://www.fmfandaid.org	Associate Member
Fondation Sanfilippo Suisse	https://www.fondation-sanfilippo.ch/	Associate Member
Fraxas - Association X Fragile Suisse	http://www.fraxas.ch	Full Member
HAEi - Hereditary Angioedema International Association	http://www.haei.org	Associate Member
HHT-Swiss	http://www.hhtswiss.org	Associate Member
MaRaVal – maladies rares valais – seltene krankheiten wallis.	https://www.maraval.ch/	Full Member
Marfan Foundation Switzerland (Marfan Stiftung Schweiz)	http://www.marfan.ch	Full Member
Prader Willi Syndrom Vereinigung Schweiz	https://www.prader-willi.ch/	Full Member
Proraris	http://www.proraris.ch	Full Member
Save sight now Europe	https://savesightnoweurope.org/	Associate Member
Schweizerische Gesellschaft Für Porphyrie	http://www.porphyria.ch	Full Member
Sjögren Europe	http://sjogreneurope.org/	Associate Member
SMA Schweiz	http://www.sma-schweiz.ch	Associate Member
Swiss FH/Schweizerische Gesellschaft für familiäre Formen der Hypercholesterinämie	http://www.sgfh.ch	Associate Member

TAIWAN, PROVINCE OF CHINA

Taiwan Foundation for Rare Disorders	https://www.tfrd.org.tw/tfrd/	Associate Member

TURKEY

DMD Aileleri Derneği (DMD Families Association)	https://www.dmdaileleri.org/	Full Member
Duchenne Kas Hastalığı Ile Mucadele Dernegi	http://dmdturkiye.org/	Full Member
Kifder	http://www.kifder.org.tr	Associate Member
Mukopolisakkaridoz ve Benzeri Lizozomal Depo Hastaliklari Dernergi / MPS Turkey	http://www.mpsturk.org	Associate Member
Pulmoner Hipertansiyon ve Skleroderma Hasta Derneği / PHA Turkey	http://www.pahssc.org.tr	Full Member
Sistinozis Hastaları Dernegi / Cıstınosıs Patıents Associatıon	https://www.sistinder.org/	Associate Member
Türkiye SMA Foundation	https://www.sma.org.tr/	Full Member
Yuzumle Mutluyum Dernegi/Happy Faces	http://www.yuzumlemutluyum.org.tr	Associate Member

UKRAINE

Association of Patients with Pulmonary Hypertension	https://pha.org.ua/ua	Full Member
Charitable Foundation Zaporuka	https://zaporuka.org.ua/	Associate Member
Children with Spinal Muscular Atrophy, Charitable Foundation	https://csma.org.ua/	Full Member
LCCF “Sister Dalila” Pulmonary Hypertension Ukrainian Rare Disease Association	https://poryatunok.info/	Full Member
Patients of Ukraine	https://patients.org.ua/en/	Associate Member

Rare Diseases of Ukraine	https://www.facebook.com/orphandisua/	Full Member
Ukrainian Association Crystal People		Associate Member
Ukrainian Association of Help for Patients with CF	http://facebook.com/cysticfibrosisua	Full Member
Ukranian Parent Project «Mio-Life»	http://miolife.org	Associate Member

UNITED KINGDOM OF GREAT BRITAIN AND NORTHERN IRELAND

Acrodysostosis Support and Research	http://www.acrodysostosis.com	Associate Member
Action Duchenne	http://www.actionduchenne.org	Full Member
Advocacy For Neuroacanthocytosis Patients	http://www.naadvocacy.org	Associate Member
AKU Society	http://www.akusociety.org	Full Member
Alex The Leukodystrophy Charity	https://www.alextlc.org/	Associate Member
Alport UK	http://www.alportuk.org/	Full Member
Alstrom Syndrome Europe	https://www.alstrom.org.uk/as-europe/	Associate Member
Alstrom Syndrome UK	http://www.alstrom.org.uk	Full Member
Amyloidosis UK	https://ttramyloidosis.uk/	Associate Member
Annabelle's Challenge	http://www.annabelleschallenge.org	Full Member
Arthur's Quest / SLC6A1 Connect UK	https://arthursquest.org/	Full Member
Association for Glycogen Storage Disease	http://www.agsd.org.uk	Full Member
Association for Multiple Endocrine Neoplasia Disorders	http://www.amend.org.uk	Full Member
Ataxia UK	https://www.ataxia.org.uk/	Full Member
Batten Disease Family Association	http://www.bdfa-uk.org.uk/	Full Member
Beacon for Rare Diseases	https://www.rarebeacon.org/	Associate Member
Behcet's UK	http://www.behcets.org.uk	Full Member
Brittle Bone Society	http://www.brittlebone.org	Associate Member
Cambridge Rare Disease Network	http://www.camraredisease.com	Associate Member
Cancer 52	http://www.cancer52.org.uk	Associate Member
CASK Research Foundation	https://caskresearch.org/	Full Member
Cavernoma Alliance UK	http://www.cavernoma.org.uk	Full Member
CDG UK	https://cdg-uk.org/	Full Member
CDH International	https://cdhi.org/	Associate Member
CDH Uk	http://www.cdhuk.org.uk/	Full Member
CHAMP1 UK	https://champ1.uk/	Associate Member
Child Growth Foundation	http://www.childgrowthfoundation.org	Full Member
Child Lung Foundation	http://www.childlungfoundation.org	Associate Member
Childhood Tumor Trust	https://www.childhoodtumourtrust.org.uk/	Full Member
Children's Liver Disease Foundation	http://www.childliverdisease.org	Associate Member
Ciliopathy Alliance	http://www.ciliopathyalliance.org	Full Member
Contact A Family	http://www.cafamily.org.uk	Full Member
CPA Research Foundation	https://www.cparfoundation.org/	Full Member
Cure CLCN4	https://cureclcn4.org/	Associate Member
Cure Myotonic Dystrophy UK Charity	https://www.congenitalmyotonicdystrophy.org/	Full Member

Cystinosis Foundation UK	http://www.cystinosis.org.uk	Full Member
Dancing Eye Syndrome Support Trust	http://www.dancingeyes.org.uk	Full Member
Ectodermal Dysplasia Society	https://edsociety.co.uk/	Full Member
EOS Network Eosinophilic Diseases Charity	https://www.eosnetwork.org/	Associate Member
European Lung Foundation	http://www.european-lung-foundation.org/	Associate Member
European Tuberous Sclerosis Complex Association	http://www.e-tsc.eu	Full Member
Eyes on the Future	https://eyesonthefuture.org.uk/	Associate Member
Fetal Anti Convulsant Syndrome Assocation		Associate Member
FOP Friends	http://www.fopfriends.com	Full Member
Gauchers Association UK	http://www.gaucher.org.uk	Full Member
Genetic Alliance UK	http://www.geneticalliance.org.uk	Full Member
Glut1 Deficieny UK	http://www.glut1deficiency.org.uk	Associate Member
HBA Support	https://www.hbasupport.org/	Associate Member
Hope for Hasti	https://www.hopeforhasti.org/	Associate Member
Hope for Hypothalamic Hamartomas UK	http://www.hopeforhh.org	Full Member
Huntington's Disease Youth Organisation	https://en.hdyo.org/	Full Member
International Brain Tumour Alliance	http://www.theibta.org	Associate Member
International Gaucher Alliance	https://gaucheralliance.org/	Full Member
International Niemann-Pick Disease Alliance	http://www.inpda.org	Full Member
International Patient Organization for Primary Immunodeficiencies	https://ipopi.org/	Full Member
International Prader-Willi Syndrome Organisation	http://www.ipwso.org	Full Member
ITP Support Association	https://www.itpsupport.org.uk	Full Member
Joining Jack	http://www.joiningjack.org	Full Member
Krabbe UK	https://www.krabbeuk.org/	Full Member
Leber's Hereditary Optic Neuropathy Society	http://www.lhonsociety.org	Full Member
LGD Alliance Europe	http://www.lgda.eu	Full Member
Max Appeal	http://www.maxappeal.org.uk	Full Member
Mebo Research	http://www.meboresearch.org	Associate Member
Metabolic Support UK	https://metabolicsupportuk.org/	Full Member
MPS Society	https://www.mpssociety.org.uk/	Full Member
MVA Society	https://mvasociety.org/	Associate Member
Myaware	https://www.myaware.org/	Full Member
Myotubular Trust	http://www.myotubulartrust.org	Full Member
Niemann-Pick UK	http://www.npuk.org	Full Member
Northern Ireland Rare Disease Partnership	https://dev.nirdp.org.uk/	Full Member
Opie Jones Foundation	https://www.opiejonesfoundation.com/	Associate Member
Pathfinders Neuromuscular Alliance	https://www.pathfindersalliance.org.uk/	Full Member
PCD Research	https://pcdresearch.org/	Associate Member
PIP-UK Poland Syndrome Support	https://pip-uk.org/	Full Member
Pitt Hopkins UK	http://pitthopkins.org.uk/	Full Member
Polycystic Kidney Disease Charity	https://pkdcharity.org.uk	Full Member
Pompe Support Network	https://pompe.uk/	Full Member

PSC Support Pseudomyxoma Survivor	http://www.pscsupport.org.uk http://www.pseudomyxomasurvivor.co.uk	Full Member Associate Member
Pten Research Foundation	https://www.ptenresearch.org/	Associate Member
Rare Autoinflammatory Conditions Community - UK	http://www.raccuk.com	Associate Member
Rare Disease Male Mental Health Support Group		Associate Member
Rare Disease UK	http://www.raredisease.org.uk	Associate Member
Reverse Rett UK	http://www.reverserett.org.uk/	Full Member
Ring 20 Research and Support UK CIO	http://ring20researchsupport.co.uk/	Associate Member
Salivary Gland Cancer UK	https://www.salivaryglandcancer.uk/	Associate Member
Schinzel-Giedion Syndrome Foundation	https://sgsfoundation.org/	Associate Member
Smile with Shiv	http://smilewithshiv.org/	Associate Member
Stargardt's Connected	https://stargardtsconnected.org.uk/	Full Member
Stiff Person Support Group	https://lizblows.wixsite.com/spsuk	Full Member
Sturge-Weber UK	http://www.sturgeweber.org.uk	Full Member
The AADC Research Trust Children's Charity	https://www.aadcresearch.org/	Full Member
The Aarskog Foundation	http://www.aarskogsyndrome foundation.co.uk	Associate Member
The Children's Hyperinsulinism Charity	http://www.hyperinsulinism.co.uk	Full Member
The Chromosome 18 Registry and Research Society (Europe)	https://chromosome18eur.org/	Associate Member
The Cure & Action For Tay-Sachs (Cats) Foundation	http://www.cats-foundation.org	Full Member
The EHE Rare Cancer Charity	https://www.ehercc.org.uk/	Full Member
The Ehlers-Danlos Society	http://ehlers-danlos.com/	Full Member
The Fragile X Society	http://www.fragilex.org.uk/	Full Member
The Maddi Foundation	https://themaddifoundation.com/	Associate Member
The PBC Foundation (UK) Ltd	https://www.pbcfoundation.org.uk/	Associate Member
Timothy Syndrome Alliance	https://timothysyndrome.org.uk/	Full Member
TTPNetwork	https://www.ttpnetwork.org.uk/	Associate Member
Tuberous Sclerosis Association	https://tuberous-sclerosis.org/	Full Member
UK Mastocytosis Support Group	https://ukmasto.org/	Full Member
Unique - Rare Chromosome Disorder Support Group	https://rarechromo.org/	Full Member
United Kingdom Thalassaemia Society	http://www.ukts.org	Full Member
Vasculitis UK (The Vasculitis Trust)	http://www.vasculitis.org.uk	Full Member
VHL UK/Ireland	https://vhl-uk-ireland.org	Full Member

UNITED STATES OF AMERICA

Alagille Syndrome Alliance	http://www.alagille.org	Associate Member
Alstrom Syndrome International	http://www.alstrom.org	Associate Member
APS Foundation of America, Inc	http://www.apsfa.org	Associate Member
Association for Creatine Deficiencies	https://creatineinfo.org/	Associate Member
BCM Families Foundation	http://www.bluecone monochromacy.org/fr/	Associate Member
CACNA1A Foundation	https://www.cacna1a.org/	Associate Member
CLOVES Syndrome Community	https://clovessyndrome.org/	Associate Member
Cure LBSL	https://www.curelbsl.org	Associate Member

Cure Mucolipidosis	https://www.curemucolipidosis.org/	Associate Member
Cure PSP	http://www.curepsp.org	Associate Member
Defeat MSA Alliance Defeat Multiple System Atrophy Alliance.	https://defeatmsa.org	Associate Member
FMD Chat	http://fmdchat.blogspot.fr/	Associate Member
GACI Global	https://gaciglobal.org	Associate Member
GBS/CIDP Foundation International	https://www.gbs-cidp.org/	Associate Member
Hypertrophic Olivary Degeneration Association	https://HODAssoc.org	Associate Member
International Foxg1 Foundation	http://foxg1.org	Associate Member
International Pemphigus & Pemphigoid Foundation	http://www.pemphigus.org	Associate Member
International Sacral Agenesis/Caudal Regression Association	http://isacra.org/	Associate Member
International WAGR Syndrome Association	http://wagr.org/	Associate Member
International Waldenstrom's Macroglobulinemia Foundation	http://www.iwmf.com	Associate Member
Malan Syndrome Foundation	https://www.malansyndrome.org/	Associate Member
MCT8-AHDS Foundation Inc.	http://www.mct8.info	Associate Member
MitoAction	https://www.mitoaction.org/	Associate Member
Myhre Syndrome Foundation	https://www.myhresyndrome.org/	Associate Member
Neuromuscular Disease Foundation	https://curegnem.org/	Associate Member
Nord National Organization for Rare Disorders	https://rarediseases.org/	Associate Member
NTM Info & Research	https://ntminfo.org/	Associate Member
Project 8p Foundation	https://project8p.org/	Associate Member
PTEN Hamartoma Tumor Syndrome Foundation	http://www.ptenfoundation.org	Associate Member
Pura Syndrome Foundation	http://www.purasyndrome.org	Associate Member
Remember The Girls	https://rememberthegirls.org/	Associate Member
Sisters' Hope Foundation	https://sistershopefoundation.com/	Associate Member
STXBP1 Foundation	https://www.stxbp1disorders.org/	Associate Member
Superficial Siderosis Research Alliance	https://ssra.livingwithss.com/	Associate Member
SYNGAP1 Foundation	https://www.syngap1foundation.org/	Associate Member
Team Titin	https://titinmyopathy.com/	Associate Member
The Cushing Support & Research Foundation	https://csrf.net/	Associate Member
The Cute Syndrome Foundation	https://www.thecutesyndrome.com/	Associate Member
The Oxalosis & Hyperoxaluria Foundation	https://ohf.org/	Associate Member
The Snyder-Robinson Foundation	https://snyder-robinson.org/	Associate Member
Usher Syndrome Coalition	http://www.usher-syndrome.org	Associate Member

PHOTO: "MY GLANCES SING A SONG"
SPINAL MUSCULAR ATROPHY (SMA) - TURKEY

URUGUAY

Asociacion Acondroplasia Uruguay	https://www.facebook.com/acondroplasia.uruguay/	Associate Member
Fundacion Uruguaya para la Investigacion de las Enfermedades Raras	http://www.fupier.org/	Associate Member

VENEZUELA, BOLIVARIAN REPUBLIC OF

Fundación Fura	https://fundacion-fura.mozello.com/	Associate Member

ZIMBABWE

Child & Youth Care, Zimbabwe	http://www.cyc.org.zw/	Associate Member

PHOTO: ERTC BARCELONA 2024

CONFERENCES & WORKSHOPS 2024

SPEAKING ENGAGEMENTS OF EURORDIS REPRESENTATIVES AT INTERNATIONAL EXTERNAL CONFERENCES

01. **European health diplomacy and sovereignty: between public health issues and powers, European Economic and Social Committee, Brussels, Belgium.**
12 January

EURORDIS Representative:

François Houÿez represented EURORDIS, peresented on Shortages of Medicines

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02. **ACT EU Clinical Trials Analytics Workshop, European Medicines Agency (EMA), Amsterdam, The Netherlands.**
25-26 January

EURORDIS Representative:

François Houÿez represented EURORDIS, discussed Patients' Expectations from Clinical Trial Registries

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03. **The Value of Health Data, Valencia, Spain.**
8 February

EURORDIS Representative:

Jelena Malinina represented EURORDIS

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04. **Rare X conference (Rare Diseases South Africa (RDSA)), Johannesburg, South Africa & Online.**
14 February

EURORDIS Representative:

François Houÿez represented EURORDIS, presented on Community Advisory Boards

05. **Workshop on Engaging with the European Health Data Space: The Paediatric Perspective, Amsterdam, The Netherlands.**
19-20 February

EURORDIS Representative:

Jelena Malinina represented EURORDIS

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06. **37th Workshop of the EURORDIS Round Table of Companies (ERTC) : Transforming rare disease foresight into action, Multistakeholder Early Dialogues to Facilitate Patient Access, Brussels, Belgium.**
21 February

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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07. **Ukraine's European Approach to Supporting People Living with Rare Diseases in the Context of Full-Scale War, Kyiv, Ukraine.**
27 February

EURORDIS Representative:

Michael Wilbur and Hanna Boiko represented EURORDIS

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08. **"The Diagnosis of Rare Diseases in Europe", Jornada de Malalties Minoritàries, Barcelona, Spain.**
28 February

EURORDIS Representative:

Rita Francisco represented EURORDIS

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09. **Exchange of views to mark Rare Disease Day, European Parliament, Brussels, Belgium.**
29 February

EURORDIS Representative:

Yann Le Cam represented EURORDIS

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10. **EMA Rare Disease Day Webinar 'Orphan Medicines Development – ask the regulator', Online.**
29 February

EURORDIS Representative:

Virginie Hivert represented EURORDIS, presenting on "Importance of rare disease development and patient engagement"

11. **Looking to the future for rare diseases in Ireland, Dublin, Ireland.**
29 February

EURORDIS Representative:

Raquel Castro represented EURORDIS, presenting on “Integrated care for people with rare diseases”

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12. **Rareboost Platform Rare Disease Day event, Izmir Biomedicine and Genome Center, Izmir, Türkiye.**
29 February

EURORDIS Representative:

Gulcin Gumus represented EURORDIS

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13. **Eurofound Expert Meeting “Informal Care and the Role of Informal Caregivers”, Online.**
8 March

EURORDIS Representative:

Gulcin Gumus represented EURORDIS

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14. **Digital Health literacy in Europe: making the digital transition in health inclusive, Brussels, Belgium.**
12 March

EURORDIS Representative:

Jelena Malinina represented EURORDIS

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15. **DIA Europe 2024, Brussels, Belgium.**
12-13 March

EURORDIS Representative:

Virginie Hivert represented EURORDIS, contributing to sessions: «How To Stimulate Orphan Drug Innovation In Europe Under The New Legislative Framework” and «Europe on the Biopharmaceutical Innovation Map”

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16. **DIA Euromeeting, Brussels, Belgium.**
13-14 March

EURORDIS Representative:

François Houÿez represented EURORDIS, presenting on «European Medicine Agencies Strategy 2028”, on “Drug Repurposing Opportunities and Challenges” and on “Timely Access to OMPs/ATMPS»

17. **Future proof-health systems: the role of SMEs, Online.**
14 March

EURORDIS Representative:

Jelena Malinina represented EURORDIS

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18. **c4c Multi-Stakeholder Meeting on paediatric Irritability, Nice, France.**
18-19 March

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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19. **Belgian Presidency Conference «Towards full inclusion of persons with disabilities – the European Pillar of Social Rights and beyond», Brussels, Belgium.**
19 March

EURORDIS Representative:

Raquel Castro represented EURORDIS

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20. **Online Eurordis Conversation on Translarna, Online.**
19 March

EURORDIS Representative:

François Houÿez represented EURORDIS, presenting on “Non-renewal marketing authorisation of Translarna”

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21. **Strategic Review and Learning Meeting of the Committee for Orphan Medicinal Products (COMP), Leuven, Belgium.**
28-29 March

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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22. **“Tests Génétiques & Molécuaires du Patient Expert Center”, Online.**
4 April

EURORDIS Representative:

Roseline Favresse represented EURORDIS

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23. **European Medicines Agency: Webinar on the Collaborare project, Online.**
4 April

EURORDIS Representative:

Maria Cavaller represented EURORDIS

24. **The Economist Impact, Brussels, Belgium.**
15-16 April

EURORDIS Representative:

Virginie Bros-Facer and Virginie Hivert represented EURORDIS

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25. **Meeting of the National Competent Authorities on Pricing and Reimbursement and Public Healthcare Payers (NCAPR), Brussels, Belgium.**
19 April

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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26. **EFA Meet & Greet with the EU training, Online.**
23 April

EURORDIS Representative:

François Houÿez represented EURORDIS, presenting on “Patient involvement in clinical development and practical implications for (decentralised) clinical trials”

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27. **Conference “Technology in employment. A step towards inclusion” & General Assembly of European Disability Forum, Ljubljana, Slovenia.**
11-12 May

EURORDIS Representative:

Claudio Pirola represented EURORDIS

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28. **2024 Trust and Health Special Report: Lunch Debate, Brussels, Belgium.**
13 May

EURORDIS Representative:

Jelena Malinina represented EURORDIS

PHOTO: “I BET IT’S TASTY... BUT I CAN ONLY IMAGINE.”
FAMILIAL CHYLOMICRONEMIA SYNDROME - ESTONIA

29. **CNA – presentation 'collaborare', Brussels, Belgium.**
14 May

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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30. **IRDIRC + RD China Congress, Shanghai, China.**
21 May

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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31. **OECD Roundtable for Legal Basis for Secondary Use of Health Data, Online.**
21 May

EURORDIS Representative:

Jelena Malinina represented EURORDIS

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32. **EJP RD Final Conference Meeting, Bari, Italy.**
27-28 May

EURORDIS Representative:

Roseline Favresse represented EURORDIS

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33. **Newborn Screening in Europe & The role of patient organizations in driving research on Newborn Screening, European Society of Human Genetics, Berlin, Germany.**
2 June

EURORDIS Representative:

Gulcin Gumus represented EURORDIS

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34. **c4c General Assembly, Bergen, Norway.**
4-6 June

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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35. **EHA2024 Hybrid Congress, Madrid, Spain.**
13 June

EURORDIS Representative:

Maria Cavaller represented EURORDIS

36. **Joint HMA/EMA Big Data Steering Group workshop on RWE methods, Amsterdam, The Netherlands.**
14 June

EURORDIS Representative:

François Houÿez represented EURORDIS, discussing the experience gained in the HTx project

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37. **HTAi annual meeting, Sevilla, Spain.**
15-17 June

EURORDIS Representative:

François Houÿez and Juljen Delaye, represented EURORDIS, organising and presenting a Workshop on the Roles of Patients in HTA

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38. **Journée PERIGENOMED sur le dépistage néonatal, Dijon, France.**
25 June

EURORDIS Representative:

Virginie Bros-Facer, Jessie Dubief, Roseline Favresse represented EURORDIS

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39. **Myeloma Patient Europe and ALAN webinar on patient and clinician involvement in the EU HTA regulation, Online.**
26 June

EURORDIS Representative:

François Houÿez, represented EURORDIS, presenting on "The new European cooperation on HTA HTAR"

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40. **ERN-RITA Patient-Centered webinar: Social rights of a person with a rare disease & patient perspective in advocacy, Online.**
26 June

EURORDIS Representative:

Raquel Castro, represented EURORDIS, presenting on "Advocating for Social rights of people with rare diseases"

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41. **European Medicines Agency (EMA) Patients' and Consumers' (PCWP) Working Party meeting, Amsterdam, The Netherlands.**
2 July

EURORDIS Representative:

Maria Cavaller represented EURORDIS

42. **Novel Medicines Platform, World Health Organization (WHO), Amsterdam, The Netherlands.**
2 July

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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43. **Leaving No One Behind: European Commitment to Rare Diseases, European Economic and Social Committee, Brussels, Belgium.**
12 July

EURORDIS Representative:

Valentina Bottarelli represented EURORDIS

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44. **ICAN Summit, Bari.**
15-18 July

EURORDIS Representative:

Maria Cavaller and Gulcin Gumus represented EURORDIS

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45. **Cancer Patients Europe, EU HTA Regulation; The role and involvement of cancer patients, Online.**
28 August

EURORDIS Representative:

Julien Delaye represented EURORDIS, presenting on “the role and involvement of patients in JCA”

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46. **BBMRI-ERIC Workshop on information points for citizens under the EHDS, Brussels, Belgium.**
5 September

EURORDIS Representative:

Jelena Malinina represented EURORDIS

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47. **Committee for Orphan Medicinal Products (COMP), European Medicines Agency (EMA), Amsterdam, The Netherlands.**
10-12 September

EURORDIS Representative:

Maria Cavaller represented EURORDIS

48. **NORBS Caring for Rare 2024: Empowering rare disease through data, Belgrade, Serbia.**
13 September

EURORDIS Representative:

Jelena Malinina represented EURORDIS

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49. **Thematic Seminars on Access to Healthcare for Persons with Disabilities, Brussels, Belgium.**
16-20 September

EURORDIS Representative:

Raquel Castro and Charissa Frank represented EURORDIS

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50. **EC, HMA & EMA multi-stakeholder workshop on Pharmacogenomics, Amsterdam, The Netherlands.**
24 September

EURORDIS Representative:

François Houÿez, represented EURORDIS, discussing "Patients' views on pharmacogenomics"

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51. **EHFG: Is the AI Act a gamechanger for healthcare?, Gastein, Austria.**
26 September

EURORDIS Representative:

Jelena Malinina represented EURORDIS

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52. **IHI Workshop on Real World Data, Digital Health and AI, Brussels, Belgium.**
1 October

EURORDIS Representative:

Gulcin Gumus represented EURORDIS

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53. **Euronews Health Summit, Brussels, Belgium.**
2 October

EURORDIS Representative:

Jelena Malinina represented EURORDIS

54. **TOPRA Annual Symposium 2024, Rotterdam, The Netherlands.**
2 October

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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55. **Université d'Automne de l'Alliance Maladies Rares, Châtillon, France.**
5 October

EURORDIS Representative:

Roseline Favresse represented EURORDIS, presenting ERDERA

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56. **Patient-Driven Partnership: Developing a Gene Panel for Actionable Conditions for Newborn Screening Using a Collaborative Approach ICONS Conference, New York, USA.**
9-10 October

EURORDIS Representative:

Gulcin Gumus represented EURORDIS

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57. **PDCO EMA informal meeting under Hungarian presidency, Budapest, Hungary.**
10 October

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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58. **CIRS workshop «Working across regulatory and HTA agencies: collaborative, work-sharing and reliance models – what are the policy implications?», London, UK.**
10 October

EURORDIS Representative:

François Houÿez, represented EURORDIS, presenting on «Evolution of collaboration and workshare in the review and assessment of medicines»

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59. **Debate at the National Academy of Pharmacy, Paris, France.**
16 October

EURORDIS Representative:

François Houÿez, represented EURORDIS, presenting on "Patient reporting of suspected adverse reactions"

60. **World Orphan Drug Congress 2024, Barcelona, Spain**
22-25 October

EURORDIS Representative:

Raquel Castro, Maria Cavaller, Rita Francisco, Avril Daly, Virginie Bros-Facer, Matt Bolz-Johnson, and Julien Delaye represented EURORDIS

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61. **591st Plenary session A European flagship initiative for health, Brussels, Belgium.**
23 October

EURORDIS Representative:

Simona Bellagambi represented EURORDIS

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62. **European Access Academy (EAA), EU HTA: Transitioning from Preparation to Implementation: Approaching the Pain Points, Rome, Italy.**
24-25 October

EURORDIS Representative:

François Houÿez, represented EURORDIS, presenting on "Conditions for Successful Joint Clinical Assessments"

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63. **1st International Communication on Health Communication, Vall d'Hebron, Barcelona, Spain.**
25 October

EURORDIS Representative:

Julien Poulain represented EURORDIS

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64. **Strategic Review and Learning Meeting of the Committee for Orphan Medicinal Products (COMP), Budapest, Hungary.**
28-29 October

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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65. **ERDERA Opening Ceremony and Kick-off meeting, Paris, France.**
28-29 October

EURORDIS Representative:

Virginie Bros-Facer and Roseline Favresse represented EURORDIS, presenting in the 'plenary session' and on 'patient engagement and involvement in research' respectively

66. **European Organisation for Research and Treatment of Cancer (EORTC) patient days, Online.**
31 October

EURORDIS Representative:

François Houÿez represented EURORDIS, presenting on "EU Cooperation on HTA: roles patients can have"

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67. **"Errance et trajectoire diagnostiques des personnes vivant avec une maladie rare en Europe" Conférence DOME, 10e Congrès de la Société Française de Médecine Prédictive et Personnalisée (SFMPP), Paris, France.**
10 October

EURORDIS Representative:

Fatoumata Faye represented EURORDIS

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68. **HMA/EMA AI Workshop, Amsterdam, the Netherlands.**
5 November

EURORDIS Representative:

Jelena Malinina represented EURORDIS

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69. **HTAR conference, Strasbourg, France.**
8 November

EURORDIS Representative:

François Houÿez represented EURORDIS, discussing "Toward renewed socio-economic interactions?"

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70. **EU Federation of Hyperlipidemias (FH) Annual Network Meeting, "How do we advance the rare lipids agenda, and collaborate more with the wider rare diseases' community? Implications of Clinical Trials/new therapies in the FCS space, new guidelines in HoFH", Online.**
9 November

EURORDIS Representative:

François Houÿez represented EURORDIS, discussing "Progresses in Clinical Trials"

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71. **Innovative Health Initiative (IHI) Brokerage event call 9, Brussels, Belgium.**
12 November

EURORDIS Representative:

François Houÿez represented EURORDIS, presenting on "A helping hand to help patients accessing medicines"

72. **c4c-S International Symposium, Amsterdam, The Netherlands.**
13-14 November

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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73. **“Rare Barometer: The survey initiative of EURORDIS - Rare Diseases Europe”, 3º Encontro do Conselho Científico da RD-Portugal, Lisbon, Portugal.**
14 November

EURORDIS Representative:

Rita Francisco represented EURORDIS

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74. **FIPRA RWE4Decisions Symposium, Brussels, Belgium.**
14 November

EURORDIS Representative:

François Houÿez represented Eurordis

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75. **ISPOR Europe 2024, Barcelona, Spain**
18 November

EURORDIS Representative:

Maria Cavaller represented EURORDIS

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76. **Politico Health Summit, Brussels, Belgium.**
19 November

EURORDIS Representative:

François Houÿez represented EURORDIS, presenting on “Preparation of the HTA cooperation”

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77. **European Commission/HADEA meeting on hospital exemptions, Brussels, Belgium.**
21 November

EURORDIS Representative:

François Houÿez represented EURORDIS

78. **EMA Media Seminar on shortages, Amsterdam, The Netherlands.**
22 November

EURORDIS Representative:

François Houÿez represented EURORDIS, presenting on “Medicines shortages and their impact on patients”

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79. **DIA Middle East & North Africa Conference, Cairo, Egypt & Online.**
27 November

EURORDIS Representative:

Virginie Hivert represented EURORDIS, presenting on ‘Patient Access to Orphan Drugs in European National Health Systems: Learned Lessons’

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80. **European Patients Forum, Data Save Lives, Brussels, Belgium.**
27 November

EURORDIS Representative:

Julien Delaye represented EURORDIS

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81. **European Day of Persons with Disabilities, Brussels, Belgium.**
28-29 November

EURORDIS Representative:

Raquel Castro and Adéla Odrihocká represented EURORDIS

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82. **For an EU commitment to tackling rare diseases, European Economic and Social Committee, Budapest, Hungary.**
29 November

EURORDIS Representative:

Virginie Bros-Facer represented EURORDIS

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83. **HTA Stakeholder Network meeting, Brussels, Belgium.**
29 November

EURORDIS Representative:

François Houÿez and Julien Delaye represented EURORDIS, presenting “ERTC workshop report” and “EUCAPA and ERTC Flash Debates” respectively

84. **CEIBS conference on International Market Access of Pharmaceuticals, Shanghai, China.**
5 December

EURORDIS Representative:

François Houÿez represented EURORDIS, discussing “Challenges and opportunities of Global Market Access for Pharmaceuticals”

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85. **ERICA General Assembly, Udine, Italy.**
11-13 December

EURORDIS Representative:

Maria Cavaller represented EURORDIS

PHOTO: “WHEN THERE'S A WILL THERE'S A WAY” DRAVET SYNDROME - SPAIN

JOURNAL PUBLICATIONS LIST 2024

01. **Drug repurposing for rare: progress and opportunities for the rare disease community, January 2024**

Contributing author:

Maria Cavaller-Bellaubi

Link:

https://www.frontiersin.org/articles/10.3389/fmed.2024.1352803/full

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02. **An Inclusive Civil Society Dialogue for Successful Implementation of the EU HTA Regulation: Call to Action to Ensure Appropriate Involvement of Stakeholders and Collaborators, March 2024**

Contributing author:

Francois Houÿez

Link:

https://doi.org/10.3390/jmahp12010004

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03. **Time to diagnosis and determinants of diagnostic delays of people living with a rare disease: results of a Rare Barometer retrospective patient survey, May 2024**

Lead authors:

Jessie Dubief & Fatoumata Faye

Contributing authors:

Michael Wilbur, Yann Le Cam

Link:

https://www.nature.com/articles/s41431-024-01604-z

04. **The European joint programme on rare diseases: building the rare diseases research ecosystem, June 2024**

Contributing author:

Roseline Favresse

Link:

https://www.oaepublish.com/articles/rdodj.2024.06?utm_campaign=website&utm_medium=email&utm_source=sendgrid.com

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05. **Drug repurposing in Rett and Rett-like syndromes: a promising yet underrated opportunity?, July 2024**

Contributing author:

Claudia Fuchs (lead author)

Link:

https://www.frontiersin.org/journals/medicine/articles/10.3389/fmed.2024.1425038/full

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06. **Framing the European Rare Diseases field through a structured movement of patient organisations, July 2024**

Contributing authors:

Maria Cavaller-Bellaubi, Virginie Hivert, Roseline Favresse and Yann Le Cam

Link:

https://www.oaepublish.com/articles/rdodj.2023.58?to=fig1

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07. **Patient Organizations: Advocating for Timely Newborn Screening & Improved Quality of Life, August 2024**

Contributing author:

Gulcin Gumus

Link:

https://www.oaepublish.com/articles/rdodj.2024.11

08. **Essentials of Translational Pediatric Drug Development, August 2024**

Chapter authors:

Ariane Weinman and Maria Cavaller

Link:

https://www.sciencedirect.com/book/9780323884594/essentials-of-translational-pediatric-drug-development#book-description

09. **The Rare Disease Moonshot: Paradigms Shift, Translational Medicine, and Regulatory Science for the World's Rarest Conditions, August 2024**

Contributing author:

Roseline Favresse

Link:

https://ascpt.onlinelibrary.wiley.com/doi/10.1002/cpt.3428

10. **A systematic review of studies that estimated the burden of chronic non-communicable diseases using disability-adjusted life-years, September 2024**

Contributing author:

Julien Delaye

Link:

https://ojrd.biomedcentral.com/articles/10.1186/s13023-024-03342-3

11. **Access in the rare diseases landscape, September 2024**

Contributing author:

Maria Cavaller-Bellaubi

Link:

https://www.sciencedirect.com/science/article/pii/S2214109X24003413

12. **Consolidated Health Economic Evaluation Reporting Standards for Interventions That Use Artificial Intelligence (CHEERS-AI), September 2024**

Contributing author:

Julien Delaye

Link:

https://ascpt.onlinelibrary.wiley.com/doi/10.1002/cpt.3428

13. **Implementing a sandbox approach in health technology assessment: benefits and recommendations, November 2024**

Contributing author:

François Houÿez

Link:

https://pmc.ncbi.nlm.nih.gov/articles/PMC11569896/

14. **Experiences of people living with a rare disease in secondary and tertiary healthcare settings: a scoping review, December 2024**

Lead author:

Rita Francisco

Corresponding author:

Jessie Dubief

Link:

https://ojrd.biomedcentral.com/articles/10.1186/s13023-024-03293-9

PHOTO: "NAVIGATION NEEDS A COMPASS"
ANGELMAN SYNDROME - AUSTRALIA

15. **Reference Centers for Rare Diseases in Portugal: How Collaborative Discussions Between Key Stakeholders Contribute to Improve Healthcare for People Living with Rare Diseases, December 2024**

Contributing author:

Rita Francisco

Link:

https://ojrd.biomedcentral.com/articles/10.1186/s13023-024-03293-9

16. **Supporting the continuous development and use of a patient partnership framework in European rare disease networks (ERNs): a scoping review of frameworks in the scientific literature, December 2024**

Contributing author:

Inés Hernando

Link:

https://doi.org/10.1007/s12687-024-00763-2

PHOTO: "BEYOND LIMITS A PIZZA WORKSHOP EXPERIENCE" SPINAL MUSCULAR ATROPHY - TURKEY

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PATIENT ORGANISATIONS AND PUBLIC ENTITIES

AFM – TÉLÉTHON

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DG Health and Food Safety

- The Operating Grant for year 2024
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- JARDIN (Joint Action)

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EUROPEAN COMMISSION
DG Research and Innovation

- The European Joint Programme on Rare Diseases (EJP RD)
- European Rare Diseases Research Alliance (ERDERA)
- European Rare Disease Research Coordination and Support Action consortium (ERICA)
- Solving the Unsolved Rare Diseases (Solve-RD)
- The Next Generation Health Technology Assessment (HTx)
- More Effectively Using Registries to suppOrt PAtient-centered Regulatory and HTA decision-making (More-EUROPA)
- REMEDi4ALL
- JOIN4ATMP
- The Innovative Health Initiative (earlier IMI2) projects:
  - conect4children (c4c)
  - Screen4Care
  - FACILITATE – FrAmework for CIInicaL trIal participants' daTA reutilization for a fully Transparent and Ethical ecosystem

Co-funded by the Horizon 2020 programme of the European Union

Co-funded by the European Union

PHOTO: "LUCA AND DALIA"
GAUCHER DISEASE - ROMANIA

HEALTH SECTOR CORPORATES

EURORDIS appreciates the contributions received from health sector companies. Ensuring a sustained variety of funding is key to minimise potential conflicts of interest. EURORDIS had 72 different corporate donors in 2024.

These companies have supported EURORDIS through the EURORDIS Round Table of Companies[1], the European Conference on Rare Diseases and Orphan Products[2], the EURORDIS Black Pearl Awards[3], as well as EURORDIS Initiatives such as Rare Disease Day[4], EURORDIS Open Academy[5], Mental Wellbeing[6], Social Policy[7] and multi-lingual communications, as well as through contributions supporting the Rare Barometer[8] programme, project development and unsolicited donations.

The breakdown of each company's contributions by project is detailed on the EURORDIS website on the "Corporate revenue" tab of the "Our Funding" section.

TOP FIVE DONORS

Takeda

01

Pfizer

02

Roche

04

Sanofi

03

Alexion – AstraZeneca Rare Disease

05

[1]**EURORDIS Round Table of Companies:**
http://www.eurordis.org/content/ertc-members

[2]**European Conference on Rare Diseases and Orphan Products:**
https://www.rare-diseases.eu/

[3]**EURORDIS Black Pearl Awards:**
https://www.eurordis.org/black-pearl-awards/

[4]**Rare Disease Day:**
https://www.rarediseaseday.org

[5]**EURORDIS Open Academy:**
https://openacademy.eurordis.org

[6]**Mental Wellbeing:**
https://www.eurordis.org/mental-wellbeing/

[7]**Social Policy:**
https://www.eurordis.org/social-policy-action-group/

[8]**Rare Barometer:**
http://www.eurordis.org/voices

OTHER PHARMACEUTICAL & BIOTECHNOLOGY COMPANIES & HEALTH SECTOR CORPORATES MAKING DONATIONS TO EURORDIS

FOUNDATIONS AND NON-FOR-PROFIT CONTRIBUTORS

Be your Possible

Chan Zuckerberg Initiative

Everylife Foundation for Rare Diseases

Fondation Ipsen, under the aegis of Fondation de France

The Marigold Foundation

SPECIAL MENTION

We also would like to sincerely thank all the generous individuals, organisations and companies from across many countries who made a gift to EURORDIS in 2024.

ACRONYMS AND DEFINITION

EURORDIS INTERNAL AND TASKFORCES

BoD	Board of Directors (of EURORDIS)
BoO	Board of Officers (of EURORDIS)
NA	National Alliance (of Rare Disease Patient Associations)
CEF	Council of European Federations of Rare Diseases
CNA	Council of National Alliances (of Rare Disease Patient Associations)
DITA	Drug, Information, Transparency & Access (Task Force of EURORDIS)
SPAG	Social Policy Action Group (Task Force of EURORDIS)
TAG	Therapeutic Action Group (of EURORDIS) – Brings together EURORDIS' representatives (mainly volunteers) in EMA scientific committees
DAG	Digital and Data Advisory Group (of EURORDIS)

EURORDIS PROJECTS/INITIATIVES (OR IN WHICH EURORDIS IS INVOLVED)

ERTC	EURORDIS Round Table of Companies (with pharma & biotech developing Orphan Medicinal Products)
OA	EURORDIS Open Academy
ePAG	European Patient Advocacy Group
ECRD	European Conference on Rare Diseases and Orphan Products
RDD	Rare Disease Day
RDI	Rare Diseases International
EMM	EURORDIS Membership Meeting
AGA	Annual General Assembly

BPA	Black Pearl Awards
RDW	Rare Disease Week
RB	Rare Barometer
Rare 2030	Rare 2030 Foresight Study
BBMRI Stakeholders' Forum	European Clinical Research Infrastructures Network
E-Rare	Network of ten partners (public bodies, ministries, and research management organisations) from eight countries, responsible for the development and management of national/regional research programs on rare diseases
EUnetHTA Forum	Support effective HTA collaboration in Europe, bringing added value at European, national, and regional levels
EuroBioBank	European Network of DNA, cell, and tissue banks for rare diseases – EU project 2003–2006, now the biobank network of RD-Connect
EUROPLAN	Fostering National Plans in Europe (project ended in 2018)
EUPATI	Innovative Medicines Initiatives Joint Undertaking – "Fostering Patient Awareness on Pharmaceutical Innovation"
FACILITATE	GDPR-compliant project in clinical trials to facilitate data sharing while respecting patient needs and adding value for stakeholders
Global Commission	Multidisciplinary expert group focused on ending the diagnostic odyssey for children with rare diseases
HTx Project	EU-funded project aiming to enhance health technology assessment (HTA)
InnovCare	Innovative Patient-Centred Approach for Social Care Provision to Complex Conditions, DG Employment and Social Innovation (EaSI), 2015–2018
IMI	Innovative Medicines Initiative
IRDiRC	International Rare Disease Research Consortium
SCOPE	Strengthening Collaboration for Operating Pharmacovigilance in Europe (SCOPE) Joint Action

SCREEN4CARE	Project to shorten the time to diagnosis and treatment for rare disease patients using newborn screening and AI tools
SOLVE-RD	EU-funded research project (2018–2022) aimed at solving unsolved rare diseases
TREAT-NMD	Translational Research in Europe – Assessment and Treatment of Neuromuscular Diseases
EJP RD	European Joint Programme for Rare Diseases

EURORDIS AND THE EUROPEAN REGULATORY NETWORK

CAT	Committee for Advanced Therapies
CHMP	Committee for Human Medicinal Products
SAG	Scientific Advisory Group at the Committee for Human Medicinal Products
COMP	Committee of Orphan Medicinal Products
EMA	European Medicines Agency
HMA	Heads of Medicines Agencies
PCWP	Patients and Consumers Working Party
PDCO	Paediatric Drugs Committee
PRAC	Pharmacovigilance and Risk Assessment Committee
SAWP	Scientific Advice Working Party
EPAR	European Public Assessment Report
SPC/SmPC	Summary of Product Characteristics
MA	Marketing Authorisation

EUROPEAN INSTITUTIONS

EP	European Parliament
EC	European Commission

MEP	Member of European Parliament
HaDEA	European Health and Digital Executive Agency
DG GROW	Directorate General for Internal Market, Industry, Entrepreneurship, and SMEs
DG SANTE	Directorate General for Health and Food Safety
DG RTD	Directorate General for Research and Innovation
JRC	Joint Research Centre (of the European Commission, based in Ispra, Italy)
CEGCC	Commission Expert Group on Cancer Control
CEGRD	Commission Experts Group on Rare Diseases (8 patients' representatives, including 2 from EURORDIS and 2 observers)
EU HPF	EU Health Policy Forum
EU HPP	EU Health Policy Platform
ESMA	European Securities and Markets Authority
NCAs	National Component Authorities of ESMA

NON-GOVERNMENTAL PARTNERS

AFM-Téléthon	French Muscular Dystrophy Association
DIA	Drug Information Association
CORD	Canadian Organization for Rare Disorders / Chinese Organization for Rare Disorders
EFPIA	European Federation of Pharmaceutical Industries and Associations
EPF	European Patients' Forum
EPPOSI	European Platform for Patients' Organisations, Science, and Industry
EuropaBio	European Association for Bioindustries
EUCOPE	European Confederation of Pharmaceutical Entrepreneurs

ESHG	European Society of Human Genetics
IAPO	International Alliance of Patients' Organizations
IFSW-Europe	International Federation of Social Workers
Inserm	French National Institute for Health and Medical Research
ISPOR	International Society for Pharmacoeconomics and Outcomes Research
MRIS	Maladies Rares Info Services (French helpline for rare diseases)
NORD	National Organization for Rare Disorders (USA) – EURORDIS' counterpart in the US
RDI	Rare Diseases International
Orphanet	Online portal for rare diseases and orphan drugs: orpha.net
NORBS	National Organisation for Rare Diseases of Serbia
HUFERDIS	Hungarian Federation of People with Rare and Congenital Diseases
San Pau	Health Management Foundation of the Hospital de la Santa Creu & Sant Pau (Barcelona)

MISCELLANEOUS TERMS

PLWRD	People Living with Rare Diseases
CoE/CE	Centre of Expertise/Excellence
ERN	European Reference Network
EU MS	EU Member State
EUNRDHL	EU Network for Rare Diseases Helplines
NP (RD)	National Plan/Programme (for Rare Diseases)
HTA	Health Technology Assessment
MAPPS	Medicine Adaptive Pathways to Patients
MoCA	Mechanism of Coordinated Access to orphan medicinal products

PACE-ERN	Partnership for Assessment of Clinical Excellence in European Reference Network
PE	Patient Engagement
CAVOD	Clinical Added Value of Orphan Drugs
OMP	Orphan Medicinal Product
Orphan Drug	Medicinal products intended for the diagnosis, prevention, or treatment of life-threatening or serious diseases that are rare
MA	Marketing Authorisation (for a medicinal product)
PV	Pharmacovigilance
ADR	Adverse Drug Reaction
CUP	Compassionate Use Programme
ATMP	Advanced Therapy Medicinal Product
NBS	Newborn Screening
NGS	Next-Generation Sequencing
UN	United Nations
CoNGO	Conference of Non-Governmental Organisations in Consultative Relationship with the United Nations
#Resolution-4Rare	Campaign to support the call for a UN resolution on addressing the challenges of persons living with rare diseases (PLWRD) and their families
PoC	Proof of Concepts
RWE	Real-World Evidence
RWD	Real-World Data

PHOTO: BLACK PEARL AWARDS 2025

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RARE DISEASES EUROPE

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