EURORDIS
RARE DISEASES EUROPE

EURORDIS.ORG

WORK PROGRAMME 2025

Cover photo: Rare is Beautiful - Homozygous familial hypercholesterolemia - United States

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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.

OUR STRATEGIC OBJECTIVES 2021-2030

A NEW EUROPEAN POLICY FRAMEWORK TO IMPROVE LIVES OF PEOPLE LIVING WITH RARE DISEASES BY 2030

PHOTO: INTERGROUP ON CANCER AND RARE DISEASES

DELIVERING ON PRIORITY AREAS:

- DIAGNOSIS
- RESEARCH POLICY AND ACTIVITIES
- HOLISTIC CARE AND SUPPORT
- HEALTHCARE
- TREATMENTS
- DATA AND DIGITAL HEALTH

PHOTO: ECRD 2024

INCLUSIVE OF ALL RARE DISEASES, ALL REGIONS, “LEAVING NO ONE BEHIND”

PHOTO: POLSAT PLUS ARENA - GDAŃSK, POLAND RARE DISEASE DAY 2024

STRATEGIC OBJECTIVE 01:

A NEW EUROPEAN POLICY FRAMEWORK TO IMPROVE LIVES OF PEOPLE LIVING WITH RARE DISEASES BY 2030

EURORDIS will pursue the following activities to reach this Strategic Objective, alongside its strategy to:

ADVOCATE | EMPOWER | PARTNER

PHOTO: INTERGROUP ON CANCER AND RARE DISEASES

PHOTO: MEP STINE BOSSE AND ENRIQUE TEROL AT THE RARE DISEASE DAY 2025 EUROPEAN PARLIAMENT EVENT.

ADVOCATE

- Advocate for a **European Action Plan for Rare Diseases**, collaborating with the 2025 and 2026 EU Council Presidencies, and engaging the European Parliament, the European Economic and Social Committee, and the European Commission.
- Participate in the conception and management of the secretariat of the newly created European Parliament's **Intergroup on Cancer and Rare Diseases**, in collaboration with the European Cancer Organisation (ECO) and SIOP Europe (SIOPE), to influence the agenda and gain support among MEPs for rare disease policy initiatives.
- Influence the successive **EU Council Presidencies** through collaboration with partners and key stakeholders to ensure rare diseases are prioritised on the agenda through key initiatives.
- Engage the **Council of National Alliances (CNA)** in advocacy through monthly calls, two meetings, and a dedicated newsletter. Equip CNA members with advocacy strategies to lead Member State involvement in EU initiatives within key strategic areas.
- Promote the development of the **WHO Assembly Resolution on Rare Diseases** and its adoption at the World Health Assembly 2025, in cooperation with RDI and other partners, supporting the preparation of a Global Plan for Rare Diseases.
- Ensure that EURORDIS' priorities are reflected in the **European Union's long-term budget**. This involves, in particular:
  - Defining EURORDIS' position on the next **Multiannual Financial Framework (MFF) for the period 2028–2035**, ensuring substantial and relevant funding for public health, research and innovation, and social programmes of the EU, with a specific focus on rare disease priorities and actions in the areas of research, healthcare, social care, and participation.

- Advocating for the inclusion of financial priorities, as outlined in **the position paper to be developed in early 2025**, with key decision-makers throughout the entire legislative and budgetary procedure.

- Continue engaging with **new key players in the European institutions** to ensure that EURORDIS' priorities are well-known and well-supported. In particular:
  - **Members of the European Parliament**, especially within the framework of the new European Parliament Intergroup on Cancer and Rare Diseases;
  - **The European Commission**, through a specific outreach programme involving the new Commissioners and relevant new leadership across all the relevant EC departments;
  - **Health attachés and permanent representations** of EU countries in Brussels.

EMPOWER

- Strengthen the **EURORDIS network of over 1,000 members**, focusing on underrepresented geographical and disease areas. Continue expanding the EURORDIS network of patient advocates and other rare disease stakeholders while enhancing engagement through bi-monthly newsletters, social media, and other communication channels.

- Coordinate over **60 volunteer patient advocates, 32 members of the Rare Cancer Advocates Network (RCAN)**, and **100 members of the Mental Health and Wellbeing Network.**

- Continue working with National Alliances through monthly online meetings and two in-person meetings of the **CNA**, aligning national alliances with EURORDIS' strategy. Strengthen the network through peer training and best-practice exchanges.

- Coordinate the **Council of European Rare Disease Federations (CEF)** and organise one meeting with the CNA.

- Continue involving CNA and CEF members in the **development and dissemination of Rare Barometer surveys**, and provide them with tailored results per country.

- Coordinate the **European Network of Rare Disease Helplines (ENRDHL)** and prepare the annual caller profile analysis.

- Host the **Black Pearl Awards 2025** in Brussels.

PARTNER

- Start organising the **13th European Conference on Rare Diseases and Orphan Products (ECRD) 2026.**
- Continue nurturing relationships with various political stakeholders (MEPs, EESC, etc.) and partners from the rare disease community to strengthen EURORDIS' advocacy efforts.

PHOTO: RARE DISEASE WEEK 2024

STRATEGIC OBJECTIVE 02:

DELIVERING ON SIX PRIORITY AREAS

EURORDIS will focus on the following key areas:

HEALTHCARE POLICY AND SERVICES	RESEARCH POLICY AND ACTIVITIES	DATA AND DIGITAL HEALTH
DEVELOPMENT AND ACCESS TO THERAPIES	DEVELOPMENT AND ACCESS TO DIAGNOSTICS	HOLISTIC CARE AND SUPPORT

PHOTO: ECRD 2024

HEALTHCARE POLICY AND SERVICES

01 06

ADVOCATE

- Engage in internal discussions to explore the potential for collecting evidence on the impact of the **Cross-Border Healthcare Directive** on people living with rare diseases and their families, contributing to EURORDIS' position for the evaluation of the legislation scheduled for 2027, and addressing policymakers' requests to consider revisions to the legislation.
- Engage with the European Commission in support of their analysis of specific measures for people living with rare diseases in cross-border care, to be adopted within the framework of the **Social Security Regulations.**
- Develop advocacy action on the financial sustainability of the European Reference Networks (ERNs), in coordination with ERN coordinators, to maintain the level of EU funding needed to sustain their core collaborative activities and Coordination teams, as part of the overall advocacy for the long-term **EU budget (MFF) 2027–2035.**
- Advocate for the **formal recognition** of the role of patient representatives and patient umbrella organisations in the ERNs.

- Advocate for improved access to **cross-border healthcare for highly specialised healthcare services** for ultra rare disease and rare complex surgical procedures with a caseload of less than 500 in the EU. Engage with Member States and other stakeholders to define and implement a model to concentrate commissioning, funding and delivery of highly specialised services for these patient populations.

- Continue to provide **tailored results from Rare Barometer surveys** to each ERN, improving awareness and consideration of the experiences, opinions, and unmet needs of people living with a rare disease within their scope of action.

- Publish and disseminate the results of the literature review conducted on existing measures of healthcare experience for people living with a rare disease and recommendations for the development of a rare disease **Patient-Reported Experience Measure.**

EMPOWER

- Support the **implementation of patient partnerships in ERNs** by working on three priority areas:
  - **ePAG guidance and support:** Engage with individual ePAGs/ERNs to provide advice on patient partnership implementation. Test and publish three tools to monitor and assess the impact and level of patient partnership implementation within the ERNs, while providing initial hands-on support to ePAGs/ERNs that want to use EURORDIS' tools and resources.
  - **Raising awareness and serving as a hub for good practices:** Use the ERN team and the Patient Partnership Hub as the primary channels to raise awareness and encourage the adoption of tools and resources. Organise a Patient Partnership Good Practice Challenge.
  - **Peer learning opportunities:** Facilitate peer learning exchanges on topics of common interest for ePAG advocates and ERN project managers.

PARTNER

- Collect data from stakeholders and partners regarding the efficacy of the **Cross-Border Healthcare Directive** to prepare actions ahead of the scheduled review.

- **Participate in the JARDIN Joint Action,** collaborating with RD patient organisations, European Federations, and National Alliances as relevant. Support the development of disease-specific care pathways by assisting patient representatives who will lead these efforts within the ERNs. Organise a Community of Practice with these leads, and develop recommendations and a toolkit to structure patient involvement in National Reference Networks or equivalent initiatives.

- Continue building and maintaining relationships with ERN coordinators and Coordination teams, signing **Supporting Partner Agreements** to formalise partnerships with the Networks.

- Track **national plan developments** through coordination with National Alliances, supporting them to engage with national policymakers and facilitating data exchange.

RESEARCH POLICY AND ACTIVITIES

02 06

ADVOCATE

- Advocate for public-private partnerships through the **RD Moonshot** initiative, pushing for the implementation of RD Moonshot recommendations on diagnosis, clinical research, and translational research. EURORDIS will contribute to RD Moonshot activities, such as framing future IHI call topics on rare diseases and participating in awareness-raising events on the value of public-private partnerships.

- Define EURORDIS' positions on the **EU Life Sciences Strategy**, including the upcoming **EU Biotech Act**. Actively engage with relevant stakeholders to gather their perspectives and ensure a comprehensive and well-informed approach. Engage with the European Commission to contribute to the Strategy and Act before their adoption and prepare to engage with EU legislators (EP, Council) as the legislative process begins.

EMPOWER

- Deliver intensive in-person training on research and therapy development, data management, ethics, and AI in rare disease research through the **EURORDIS Open Academy and under the training work package of ERDERA (European Rare Diseases Research Alliance).**

- Develop a **beginner-level online** course under the EURORDIS Open Academy, targeted at learners joining the rare disease research ecosystem. The course content will be free and on demand, allowing learners to sign up and complete it at their own pace.

PARTNER

- Engage with the **International Consortium on Newborn Sequencing**, contributing to global newborn genomics research, in line with the Health Commissioner's mission letter.

- Collaborate with the **European Society of Human Genetics** as members of the Education Committee and Policy and Ethics Committee (PEC). EURORDIS will contribute to the PEC workshop in January on "Genomics and Justice."

- Continue active participation in the **Together4RD** initiative, supporting collaboration between ERNs and industry.

- Finalise the EU project **ERICA**, including leading a deliverable on the **'Patient Partnership Framework in Clinical Trials for the ERNs,'** designed to guide the implementation of patient partnerships at all stages of clinical trials conducted within the ERNs.

- Participate in **European Rare Diseases Research Alliance (ERDERA)** activities. EURORDIS will be an important contributor to ERDERA by being an active member of the Operating Group, co-leading one of the 7 workstreams (on Education and Training) and being active in **nine of the 25 work packages** with a total of more than four full time equivalents from EURORDIS involved. Notably, EURORDIS will be:
  - Coordinating the **Patient and Public Involvement and Engagement (PPIE)** task to work with patients and the public to shape and engage in research.
  - Co-leading the task on **'Engagement of patients in research project funding'** in Joint Transnational Calls.
  - Contributing to the launch and dissemination of the funding mechanism **"Networking to share knowledge on research."**
  - Estimating the **socioeconomic impact of rare diseases** to inform decision-making and improve resource allocation by unveiling the hidden burden of rare diseases.
  - Conducting a scoping review, stakeholder survey, and workshop to recommend criteria for selecting rare conditions suitable for **ATMP therapies.**
  - Coordinating the **Education and Training Work Stream in ERDERA.**
  - Coordinating the development, delivery, and follow-up of training programmes for patients and young researchers through the **Open Academy programme.**
  - Contributing to a repository of all relevant rare disease research training courses to be hosted on the ERDERA website and developing a **University Diploma** on rare disease research.
  - Supporting the **ERDERA Acceleration Hub** to address unmet needs and improve the lives of people living with rare diseases.
  - Supporting patient community involvement in **ERDERA National Mirror Groups**, in national activities in underrepresented countries, and in widening participation.

- Finalise the EU Project **Conect4Children (c4c)**, a large collaborative European network that aims to facilitate the development of new drugs and therapies for the paediatric population.

- Participate in the **Undiagnosed Diseases Network International (UDNI)** and co-coordinate the UDNI Patient Engagement Working Group. Coordinate activities between the JARDIN Joint Action on integrating ERNs into national health systems and the national reference networks or undiagnosed disease programmes interlinked with ERN and UDNI actions in the EU.

PHOTO: THE LAUNCH OF ERDERA 2024

DATA AND DIGITAL HEALTH

03 06

ADVOCATE

- Explore the feasibility for the CNA to support the national implementation of the **European Health Data Space (EHDS)** legislation designed to optimise electronic health records and support the ethical use of data for research and policy development.
- Represent people living with rare diseases at the European Medicines Agency's **Network Data Steering Group**, as EURORDIS' appointed representative.

EMPOWER

- Enhance digital health literacy among patient communities, focusing on consent and data usage training. Continue with a social media campaign to increase understanding of the opportunities provided by the EHDS. Deliver a **six-month online training on data management, ethics, and AI in rare disease research** through the Open Academy under ERDERA.
- Coordinate the **Digital and Data Advisory Group (DAG)** to inform EURORDIS' policy and support activities in the FACILITATE and Screen4Care projects.

PHOTO: OPEN ACADEMY 2024

PARTNER

- Participate in the IMI project **FACILITATE**, which ends in 2025, aimed at enabling clinical trial participants to access their data gathered during studies and managing an ethical and transparent process to reuse these data in future research. Take part in the annual meeting and provide three consultations with the DAG. A final FACILITATE webinar, with DAG representatives playing a central role, will take place towards the end of the year.

- Analyse the results of semi-structured interviews conducted with **patient-registry holders** to identify organisational, technical, and legal barriers to making registries interoperable with ERN registries and other national health IT systems at the Member State level, through the JARDIN Joint Action.

DEVELOPMENT AND ACCESS TO THERAPIES

04 06

ADVOCATE

- Continue engaging in the revision of the **General Pharmaceutical Legislation**, focusing on the **revisions to the Regulation on Orphan Medicinal Products and Paediatric Medicines**, to establish an EU regulatory framework that improves the development of and access to orphan medicines. Engage with Member States' representatives before the EU Council position is defined, and closely follow the negotiations between the three EU Institutions at the end of the legislative process (Trilogues) to secure balanced outcomes for the benefit of people living with rare diseases.

- Review and redefine, where appropriate, EURORDIS' positions on medicine shortages, supply chains, stockpiling, and EU manufacturing capacities, drawing from the conclusions published by the EU Critical Medicines Alliance. Propose and draft targeted amendments to the European Commission's proposal on the **EU Critical Medicines Act** to advocate for the rare disease community's specific needs (expected early 2025).

- Work towards better clinical trials organised in the EU and contribute to **ACT-EU (Accelerating Clinical Trials in the EU).**

- Call for more **treatment alternatives for rare diseases**, including ATMPs and repurposing approaches.

- Represent patient organisations on the **European Medicines Agency Management Board**, including serving as chair of the MBARG (Management Board Audit and Risks Group). Continue advocating for secure and adequate patient engagement at the Agency within the revision of the General Pharmaceutical Legislation.

- Build advocacy messages around the possible revision and implementation of the **Medical Devices Regulation and In-Vitro Diagnostic (IVD) Regulation**, focusing on Orphan Devices and Orphan IVDs.

- Advocate for improved and equitable access to rare disease treatments, promoting structured cooperation between European countries on pricing, reimbursement, and funding mechanisms. Engage with the **WHO/Europe Novel Medicines Platform (2023–2026)**, where EURORDIS participates as Vice Chair of the Working Group on Solidarity and as a member of Working Group on Sustainability.

- Develop a position on **hospital exemption**, following the publication of the European Commission's study on the topic.

EMPOWER

- Deliver **blended training** (covering research, diagnostics, medicines development, clinical research, regulatory affairs, and access) through the **Open Academy.**

- Complete the **EUCAPA project**, which trains patients and their representatives in Health Technology Assessment (HTA), and explore opportunities for continued access to these training materials. Advocate for the continuation of educational projects for patients and representatives on HTA.

- Develop and disseminate **monthly therapeutic reports** to all EURORDIS members.

- Continue participation and support patient and ERN clinicians' engagement in the **Mechanism of Coordinated Access to Orphan Medicinal Products (MoCA).**

- Support patient involvement in Health Technology Assessment (HTA) activities through the EURORDIS **HTA Task Force** and the EUCAPA project on HTA training (which ends in February 2025).

- Support patient involvement in developing quality information on medicines through the coordination of the **EURORDIS Drug Information, Transparency and Access Task Force (DITA Task Force).**

- Co-lead the **CollaboRARE pilot project** within the COMP, in partnership with the EMA stakeholder engagement department. CollaboRARE will capture patient experience data through AI-based technology, to be validated by patient organisations and used to support regulatory decision-making.

- Support **patient engagement in EMA activities** by involving patients in Scientific Advice/Protocol Assistance and in the body's committees and working parties, including the PCWP (Patients & Consumers Working Party), COMP (Committee for Orphan Medicinal Products), PDCO (Paediatric Committee), CAT (Committee for Advanced Therapies), and PRAC (Pharmacovigilance and Risk Assessment Committee). Facilitate early interactions between patient organisations and CHMP (Committee for Human Medicinal Products).

PARTNER

- Participate in the EU project **JOIN4ATMP**, aimed at accelerating and de-risking European ATMP development and ensuring widespread access to ATMPs.
- Participate in the EU project **REMEDi4ALL**, which aims to build and establish a sustainable European innovation platform to enhance the repurposing of medicines for all.
- Launch and contribute to the **IHI project RealiseD**, starting in 2025, which aims to enhance clinical trials for rare (and ultra-rare) diseases through a collaborative, patient-centred approach.
- Implement good practices for patient engagement, building on previous projects such as **IMI PARADIGM**, creating Patient Advisory Groups, and linking with ERNs and ePAGs involved in the project. Organise multistakeholder meetings to address challenges and provide recommendations.
- Participate in the EU project **More-EUROPA**, aimed at more effectively using registries to support patient-centred regulatory and HTA decision-making.
- Participate in the **IRDiRC Consortium** as a full member and expert contributor to the IRDiRC Therapeutic Scientific Committee, leading a working group on paediatric patient engagement and participating in relevant task forces and working groups, according to topic relevance.
- Work with the **GetReal Institute**, an independent, multi-stakeholder European forum that promotes the adoption and implementation of real-world evidence (RWE) in regulatory, health technology assessment, and clinical decision-making.
- Advise the **DeCODe project on Advancing Paediatric and Orphan Medical Devices** as a member of its Scientific Advisory Board.

PHOTO: "LOOK FURTHER"
RARE GENETIC DISEASE GABRB3 GENE MUTATION - ITALY

DEVELOPMENT AND ACCESS TO DIAGNOSTICS

05 06

ADVOCATE

- **Advocate for reduced diagnostic delays** for rare diseases, promoting a harmonised European approach to newborn screening based on our **11 Key Principles.**
- Push for **EU-level cooperation initiatives** and call for the **creation of an EU multistakeholder expert group** to develop this approach, working with Member States with the support of National Alliances.

- Engage with **Members of the European Parliament** to apply political pressure, possibly via the Cancer & Rare Diseases European Parliament Intergroup.
- Explore the viability of a new **NBS consortium of stakeholders** to help shape the future of newborn screening policy.
- Continue the work of the **Newborn Screening Working Group (NBS WG).** Assess the feasibility of developing actionable recommendations based on the 11 Key Principles.
- Broadly disseminate findings from the **EURORDIS Rare Barometer survey** on the diagnostic odyssey of people living with rare diseases, as well as from the Rare Barometer & Screen4Care survey on public opinion regarding newborn screening.
- Investigate the **European Commission's potential proposals to scale up genome sequencing**, as specified in the mission letter of the EU Health Commissioner. Explore the possibility to develop EU-wide initiatives informing, educating and engaging into a public dialogue to understand society's concerns around the introduction of genomic at newborn stage.

EMPOWER

- Continue mapping out newborn screening (NBS) practices through the Newborn Screening Working Group (NBS WG). As a member of the **International Consortium on Newborn Sequencing (ICoNS),** EURORDIS will support newborn genomics research to advance genetic newborn screening research and practices.
- Empower EURORDIS members and ERNs by providing access to the **Rare Barometer survey results** on the diagnostic odyssey of people living with rare diseases, as well as the Rare Barometer & Screen4Care survey on public opinion regarding newborn screening.

PARTNER

- Participate in the **IMI project Screen4Care** by coordinating Screen4Care NBS Forum activities and overseeing the clinical implementation of **newborn screening (NBS) pilots** in Italy, Germany, and France. This includes evaluating how information is provided to parents, how it is reported and analysed, and ensuring patient voices and perspectives are incorporated where necessary. Additionally, contribute to the final design of the meta-symptom checker application and the development of the virtual platform.

HOLISTIC CARE AND SUPPORT

06 06

ADVOCATE

- Continue advocating for **holistic care and support** as a priority in the **European Action Plan for Rare Diseases.**
- Promote the **integration of health and social care** for people living with rare diseases by supporting the development of case management guidelines within the Joint Action JARDIN.
- Advocate for access to **disability rights and independent living support** for people with rare diseases as a member of the EU Disability Platform and the European Disability Forum. Contribute to EU consultations on the next phase of the **European Strategy for the Rights of Persons with Disabilities 2021–2030,** ensuring that the rare disease community is not left behind.
- Communicate findings from the **Rare Barometer survey on Social Participation and Independent Living** to raise awareness of the barriers and disabilities faced by people living with rare diseases and advocate for better recognition of their disabilities and improved access to their rights.
- Push for improved access to quality and adequate social and employment rights for people with rare diseases by contributing to consultations on EU initiatives and legislative proposals under the **European Pillar of Social Rights,** in cooperation with the Social Platform. Support the development of **EU Guidelines for Social Services of Excellence** via the Disability Platform.
- Conduct a new **Rare Barometer survey on Mental Health and Wellbeing** and broadly disseminate its findings.

EMPOWER

- Support the **EURORDIS Social Policy Action Group (SPAG)** in ensuring the involvement of people with rare diseases in shaping policies and practices that improve holistic care and support, while protecting their social and human rights.

PHOTO: MENTAL HEALTH PANEL. ECRD 2024

- Provide tailored results from the **Rare Barometer surveys on Social Participation and Independent Living,** as well as the **Mental Health and Wellbeing survey,** to EURORDIS members and ERNs.
- Assist the National Alliances in engaging with the **transposition of the Directives establishing the European Disability Card,** ensuring that people with rare diseases and disabilities are eligible.
- Support the **Mental Wellbeing Partnership Network** in raising awareness of the impact of rare diseases on mental health by publishing personal testimonies, hosting webinars, and producing a revised position paper based on the Rare Barometer survey on mental health. Advocate for the recognition of the mental health needs of the rare disease community in national plans and European policy.
- Build the capacity of patient groups to strengthen the resilience of the rare disease community by developing a **mental health toolkit** and running a **peer learning workshop** at EURORDIS EMM 2025.

PARTNER

- Collaborate with the **Social Platform and the European Disability Forum** to help shape social and disability policies relevant to the rare disease community.

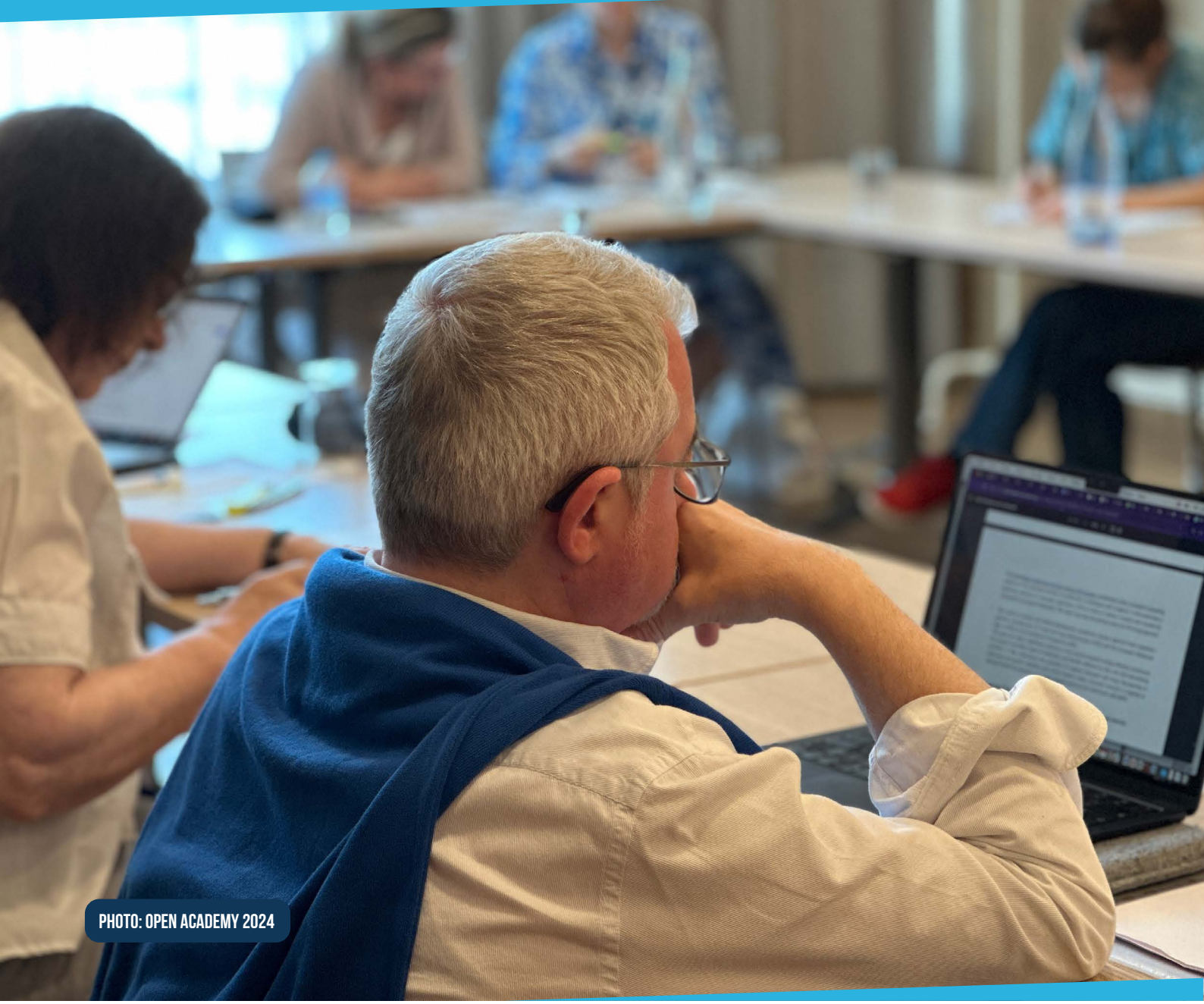


PHOTO: OPEN ACADEMY 2024

- Work with ERNs through the Joint Action JARDIN to develop **case management guidelines** for people living with rare diseases, supporting ERNs' efforts to promote integrated health and social care pathways. Leveraging EURORDIS' experience with the INNOVCare project, assist in recruiting a multi-stakeholder drafting group and organising a cross-national workshop to gather input from a wider group of experts.

- Collaborate closely with research communities and industry partners to **increase awareness of mental health considerations** throughout research activities and the therapeutic development pathway.

- Provide input to the European Commission on the **design of future funding instruments,** drawing on EURORDIS' experience with previous reviews of the Employment and Social Innovation (EaSI) programme.

- Identify and seize opportunities to develop project **proposals addressing key holistic care and social priorities,** particularly through relevant EU funding instruments.

STRATEGIC OBJECTIVE 03:

INCLUSIVE OF ALL RARE DISEASES, ALL REGIONS, “LEAVING NO ONE BEHIND”

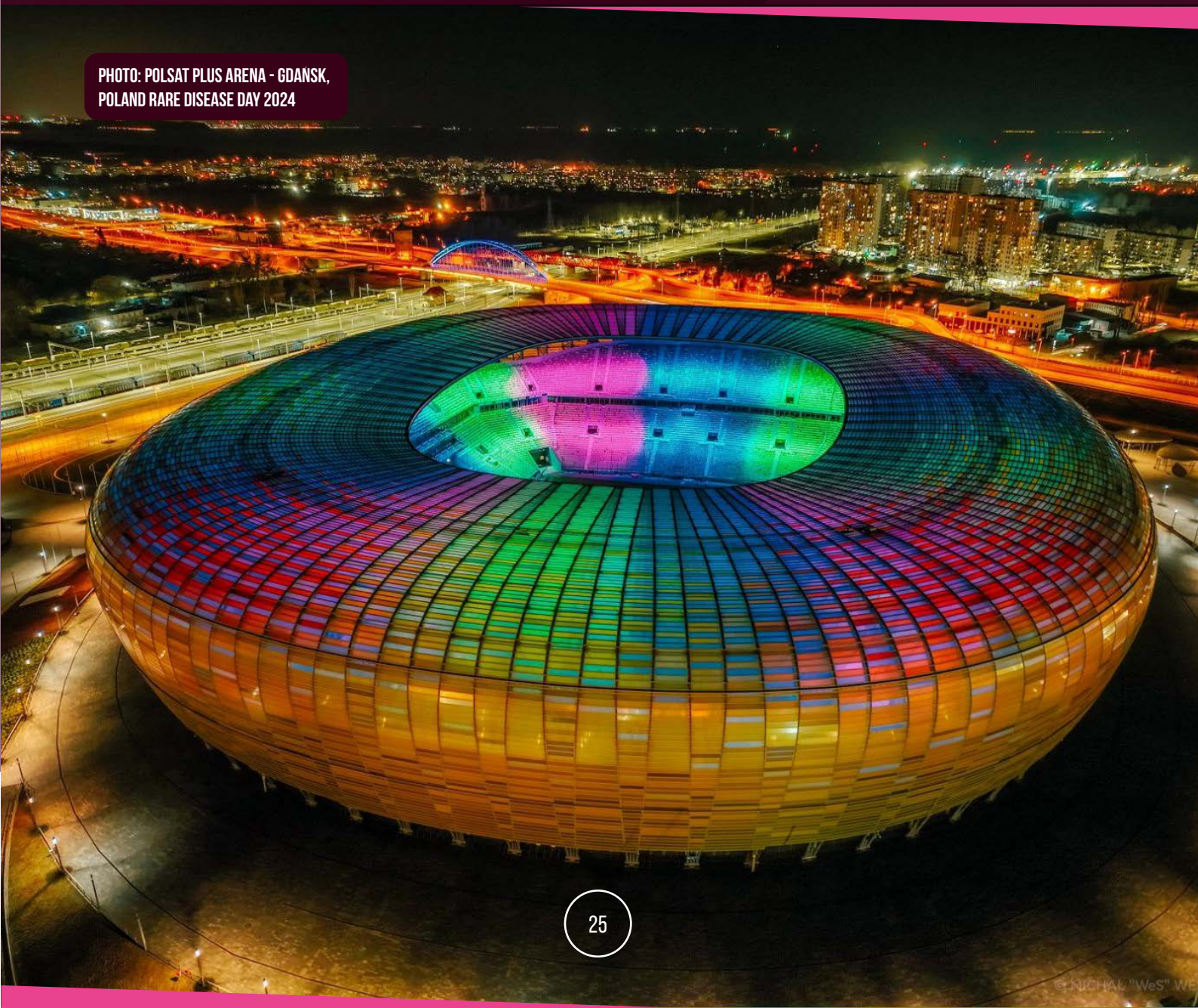


PHOTO: POLSAT PLUS ARENA - GDANSK, POLAND RARE DISEASE DAY 2024

ADVOCATE

- Increase visibility for **rare cancers,** integrating them into advocacy activities, including contributions to EU pharmaceutical legislation and national cancer plans, with support from the **RCAN.**
- Campaign with RCAN for the inclusion of paediatric cancers and rare adult cancers (as distinct sections) in **National Cancer Control Plans** in European countries, highlighting synergies with National Rare Disease Plans, where relevant.
- Advance **tailored solutions for the rarest diseases according to prevalence,** in the context of the revision of the EU Regulation on Orphan Medicinal Products, clinical trial design, access to advanced therapies, and specialised services through ERNs.

EMPOWER

- Support the **RCAN** to advance advocacy for rare cancers and collaborate on shared issues impacting all rare disease patients.
- Continue supporting **Ukrainians living with rare diseases** through the JARDIN Joint Action.
- Enhance and expand the **Open Academy** by maintaining and improving existing online courses. Translate ten of the most popular Open Academy courses into **Turkish.** Engage Open Academy alumni through newsletters, webinars, and mentoring opportunities, supporting their transition from trainees to active alumni.

PARTNER

- Collaborate in the field of **rare cancers** with patient organisations, ERNs relevant to rare cancers, WECAN (Workgroup of European Cancer patient Advocacy Networks), ECO (European Cancer Organisation), the ESMO Rare Cancers Working Group, SIOPE (the medical society for paediatric cancers), and the EHA (European Hematology Association).
- Contribute to the **RDI working group on the WHO Essential Medicines List** to improve global access to therapies for people living with rare diseases.
- Coordinate **Rare Disease Day (RDD) 2025** and prepare for **RDD 2026** in collaboration with international partners.

CROSS-CUTTING PRIORITIES:

SUPPORTING STRATEGIC OBJECTIVES

EURORDIS will support the Strategic Objectives in four areas:

- COMMUNICATION AND DISSEMINATION
- PEOPLE (STAFF AND VOLUNTEERS)
- GOVERNANCE
- RESOURCE DEVELOPMENT AND SUSTAINABILITY

PHOTO: «ELIJO LA VIDA» MARFAN SYNDROME, SPAIN

COMMUNICATION AND DISSEMINATION

04

In 2025, our communications will focus on enhancing **engagement, accessibility, and efficiency** through a series of targeted activities and initiatives:

- Maintain a communication strategy that aligns with EURORDIS' goals, optimising digital content accessibility and strengthening network engagement across platforms. Prioritise **website optimisation** to meet accessibility requirements, ensuring our digital platforms are inclusive and user-friendly for all audiences.

- Expand **multimedia initiatives** to amplify key voices within the rare disease community. Plan to release:
  - Six episodes of the **Rare on Air podcast**, showcasing patient stories.
  - Six **10 Minutes With LinkedIn Live** interviews, highlighting insights from key stakeholders.
  - Six to ten **Rare on Air Stories episodes** as part of the bonus podcast series, featuring submissions from the Rare Disease Day website to foster connections and raise awareness globally.

- Leverage **social media and newsletters** to expand outreach:
  - **Monthly LinkedIn newsletters** will focus on advocacy and news updates.
  - **Quarterly social media campaigns** will drive awareness and engagement around key initiatives and priorities.
  - **Tailored communication support** will be provided for major events, including the **Black Pearl Awards (BPA)** and other critical projects.

- Support key advocacy actions with targeted **communication strategies and campaigns,** particularly aimed at policymakers.

- **Enhance membership engagement by:**
  - Implementing a structured **induction programme.**
  - Exploring a **mentorship initiative** to support smaller and younger patient organisations.
  - Updating **Member News,** our bi-weekly newsletter, to enhance accessibility in **Polish, Ukrainian, Romanian, and Turkish,** aligning with EURORDIS' Strategic Objective to "leave no one behind."

- Improve internal efficiency by launching **OurNet,** a dedicated intranet platform to enhance internal communication and resource sharing. Additionally, we will develop **"How-To" guides** to equip staff with practical tools for streamlining internal processes and daily operations.

PHOTO: JULIEN POULAIN, HOST OF THE RARE ON AIR PODCAST

- Deliver comprehensive global communication support for **Rare Disease Day (RDD)**, including:
  - The creation of key materials, such as the **feature video and poster.**
  - **Webinars and storytelling initiatives** to maximise visibility and impact.
- Explore a potential **rebranding project** to ensure EURORDIS' visual identity aligns with our evolving goals and ambitions.

PEOPLE (STAFF AND VOLUNTEERS) 02 04

- Continue to support volunteers and enforce a good environment of work for **EURORDIS staff.**

GOVERNANCE 03 04

- Strengthen EURORDIS' governance and accountability by adopting **new good practices** where necessary and regularly **updating governance policies and by-laws.**
- **Provide governance training** to key staff and board members as needed.
- Conduct a **mid-term review** of the EURORDIS Strategy 2021–2030.
- Strengthen **existing partnerships,** including with **AFM-Téléthon,** while pursuing **multi-year funding strategies** and forging **new partnerships** where beneficial.

RESOURCE DEVELOPMENT AND SUSTAINABILITY

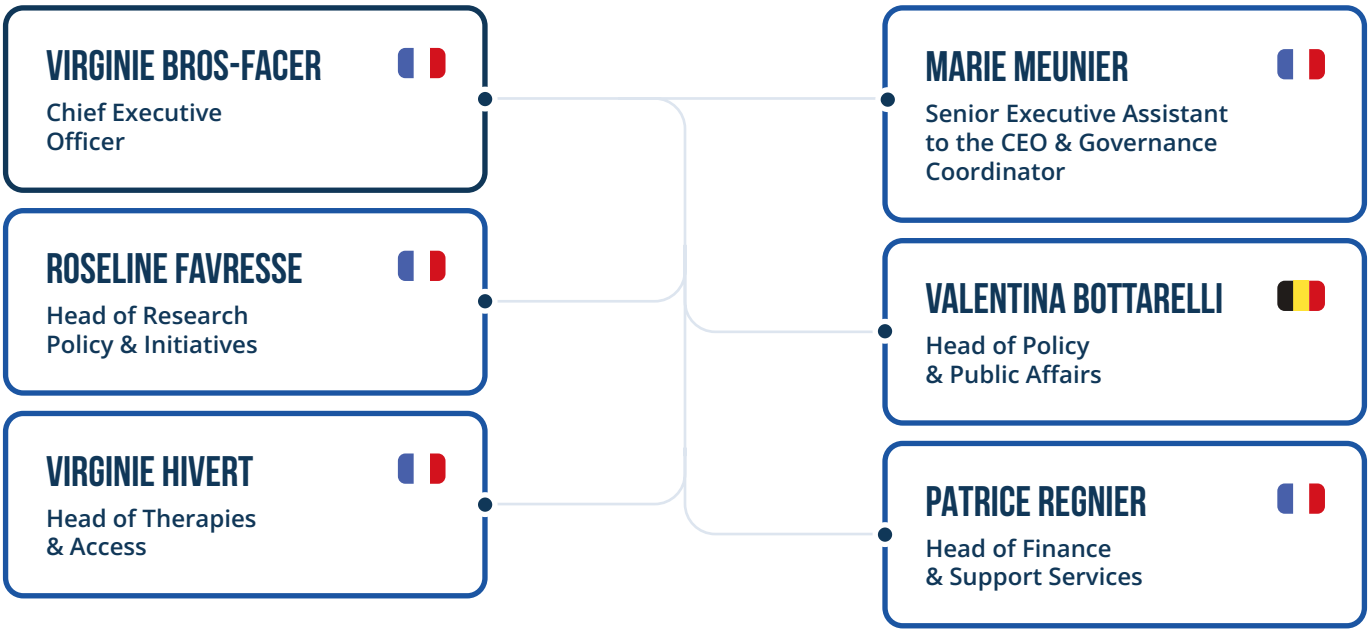
04

- Strengthen the **EURORDIS Round Table of Companies,** engaging over 70 health sector companies through regular webinars and two workshops.
- Maintain efforts to support **current contributions from the health sector.**
- Diversify funding sources, pursuing **new funding opportunities** from foundations and donors while expanding partnerships to support EURORDIS' strategic goals.

PHOTO: ERDERA LAUNCH 2024

TEAM CHART 2025

CORE LEADERSHIP TEAM



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GOVERNANCE, COMMUNICATION, EVENTS & RESOURCE DEVELOPMENT

VIRGINIE BROS-FACER
Chief Executive Officer

MARIE MEUNIER
Senior Executive Assistant to the CEO & Governance Coordinator
CLT

PATRICE REGNIER
Head of Finance & Support Services
CLT

VALENTINA BOTTARELLI
Head of Policy & Public Affairs
CLT

VIRGINIE HIVERT
Head of Therapies & Access
CLT

ROSELINE FAVRESSE
Head of Research Policy & Initiatives
CLT

ANJA HELM
Senior Manager of Relations with Patient Organisations

MARTINA BERGNA
Events Senior Manager

JESSICA KNOLLYS
Events Junior Manager

STANISLAV OSTAPENKO
Communications Director

JULIEN POULAIN
Communications Manager

RHIANNON WALLS
Communications Manager

CELINE SCHWOB
Corporate Relations Director

JO MARSHALL
Corporate & Donor Relations Manager

ANNE-MARY BODIN
Resource Development Assistant

ANDREA OSVOLL
Corporate Relations Manager

PHOTO: "FEELING THE GRASS"
CHUNG JANSEN SYNDROME - UK

RESEARCH POLICY & INITIATIVES

ROSELINE FAVRESSE
Head of Research Policy & Initiatives
CLT

GULCIN GUMUS
Research & Policy Senior Manager

VERONICA POPA
Patient Engagement Manager - Digital & Data

RITA FRANCISCO
Patient Engagement Manager (Research projects)

ZOE ALAHOUZOU
Senior Research Manager

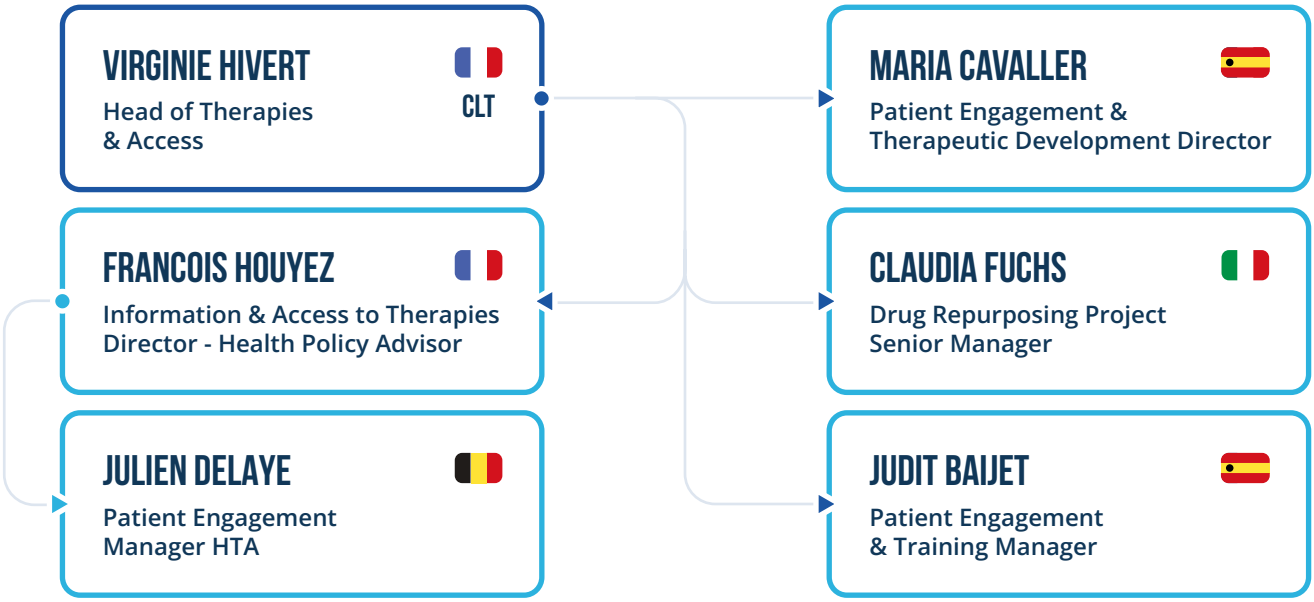
JESSIE DUBIEF
Social Research Director - Rare Barometer Programme Lead

FATOUMATA FAYE
Survey Project Manager

MARTA CAMPABADAL
Open Academy Senior Manager

GEMMA RODRÍGUEZ FABIÀ
Open Academy Training Manager

THERAPIES & ACCESS



FINANCE & SUPPORT SERVICES

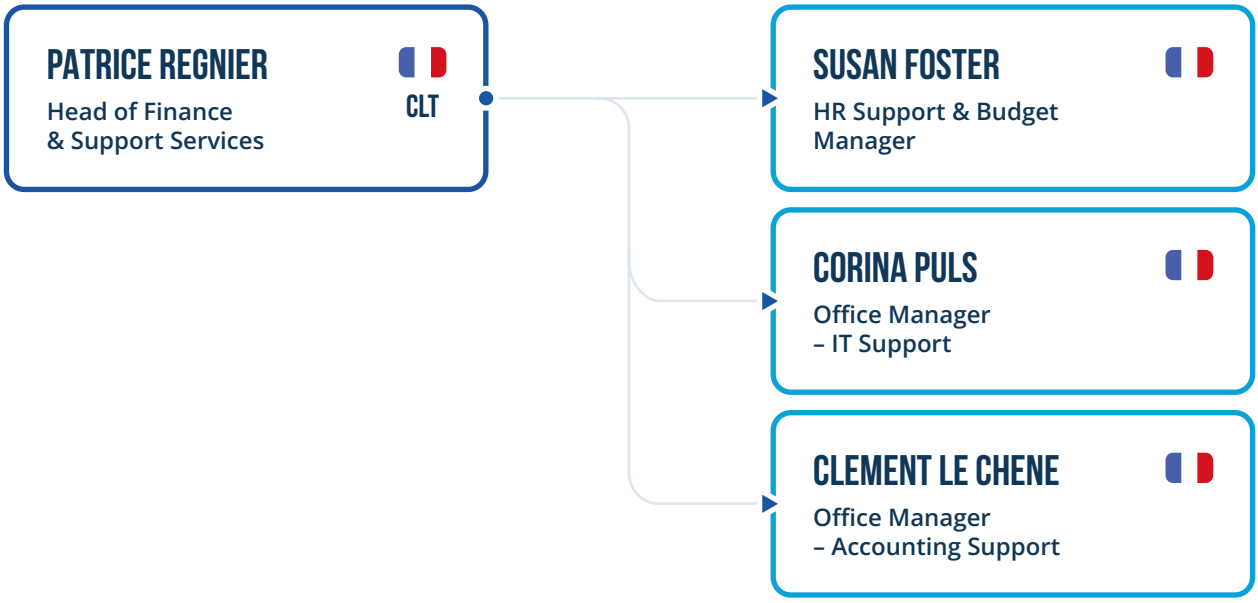


PHOTO: "WONDER AND LIGHT" ORNITHINE TRANSCARBAMYLASE DEFICIENCY, A UREA CYCLE DISORDER - USA

POLICY & PUBLIC AFFAIRS

VALENTINA BOTTARELLI
Head of Policy & Public Affairs
CLT

ELIAS BARTH
Office Manager – Team Assistant

RAQUEL CASTRO
Social Policy & Initiatives Director

ARIANE WEINMAN
Public Affairs Senior Manager – Rare Cancers

MATT JOHNSON
ATMP & Mental Wellbeing Advisor

TBR
Public Affairs Advisor

TBR
EU Public Affairs Manager

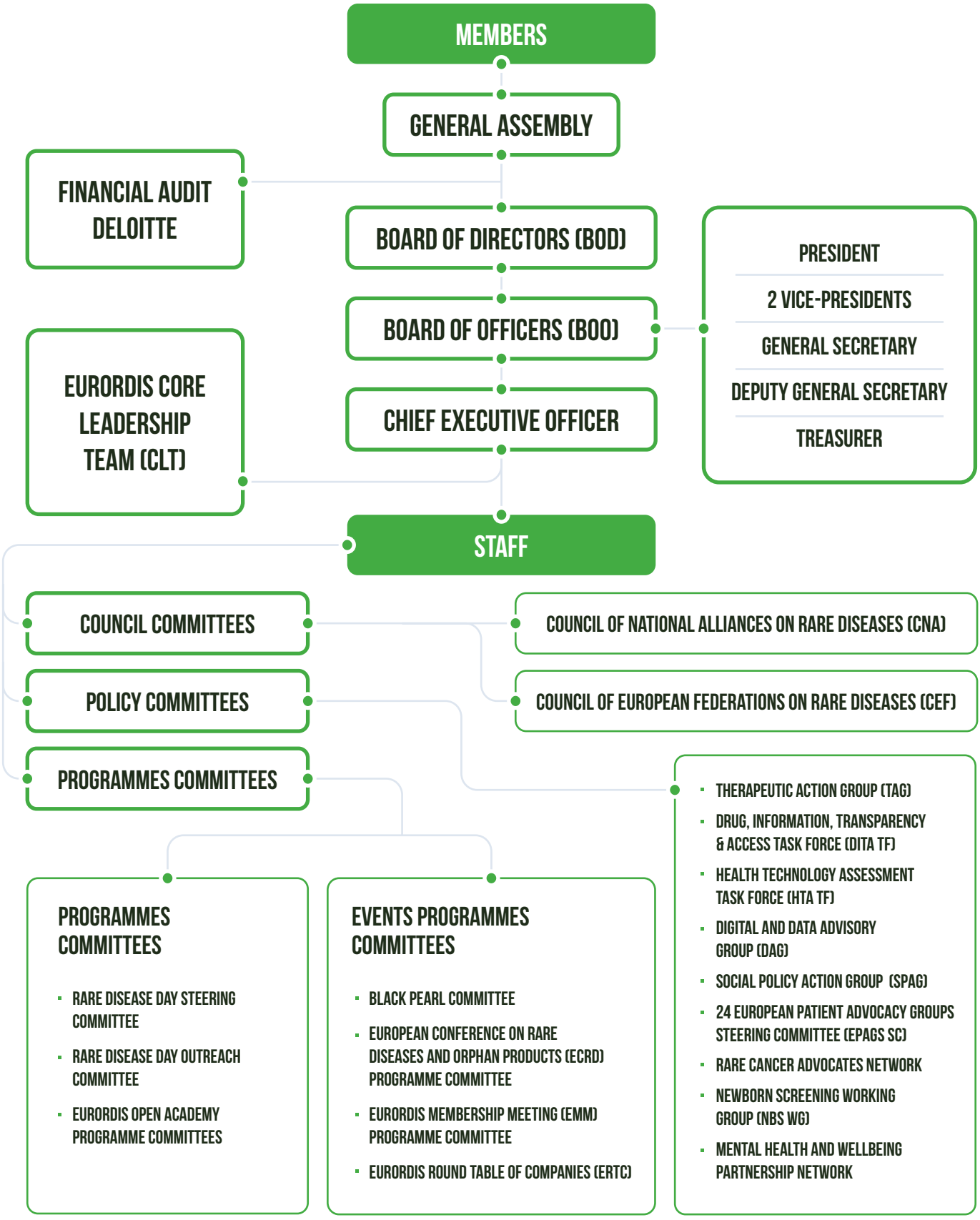
RACHEL BUTCHER
Public Affairs Manager

INES HERNANDO
Healthcare & ERN Director

ANDREA CEMBRERO BONET
Patient Engagement Manager – Healthcare

NORA LAZARO
PE Manager – Knowledge Management & Outreach

EURORDIS GOVERNANCE CHART 2025



EURORDIS' REPRESENTATION IN EXTERNAL NETWORKS, ORGANISATIONS AND INSTITUTIONS IN 2025

European Regulatory Network EMA and HMA

- MB - Management Board
- MBARG, the Management Board Audits and Risks Group
- COMP - Committee for Orphan Medicinal Products
- PDCO - Paediatric Committee
- EnprEMA - European Network of Pediatric Research EMA Coordinating Group
- CAT - Committee for Advanced Therapies
- SAWP - Scientific Advice Working Party
- PRAC - Pharmacovigilance Risk Assessment Committee
- PCWP - Patients' & Consumers' Working Party
- CTIS - Clinical Trial Information System Stakeholder and expert Group
- Advisory Group on Real World Evidence
- Topic Group on Patient Experience Data
- Network Data Steering Group (NDSG)
- ACT-EU Accelerating Clinical Trials in the EU - stakeholder platform (with EMA & HMA)

European Commission

EU Health Policy Platform

Stakeholder Network for Rare Diseases

eHealth Stakeholder group

EU Disability Platform

Critical Medicines Alliance - HERA, preparedness

Mechanism of Coordinated Access to orphan medicinal products (MoCA)

EU Cooperation on Health Technology Assessment (HTA)

Health Technology Assessment Stakeholder Network

European Parliament

EP Intergroup on cancers and rare diseases

European Reference Networks (ERNs) via support to ePAGs and/or collaboration with ERNs in other activities and projects.

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

EURORDIS' REPRESENTATION, PARTNERSHIP AND/OR SUPPORT TO NETWORKS AND ORGANISATIONS IN 2025

Member of European Not-for-Profit Organisations & Initiatives:

- EPF: European Patients' Forum (founding member) and EPF ehealth expert group
- EU4Health Civil Society Alliance (founding member)
- WECAN: Workgroup of European Cancer Patient Advocacy Networks
- ECO PAC: European Cancer Organisation Patient Advisory Committee
- European Disability Forum (EDF)
- Social Platform – European Platform of European Social NGOs
- Mental Health Europe (MHE)
- RareResourceNet - European Network of Resource Centres (Board member)
- European Network of Rare Diseases Helplines (ENRDHL, founding member and coordinator)
- European Forum for Good Clinical Practice (EFGCP)
- Friends of Europe (Think Tank, European policy)
- FIPRA – International Policy Advisors
- Rare Disease Platform in Paris (founding member)
- Maladies Rares Info Service - French Helpline for RDs (Board member)
- 4th French Rare Disease National Plan Steering Committee
- Italian Rare Diseases Plan Steering Committee
- Get Real Institute (founding member)
- TRANSFORM

Partnering with European Not-for-Profit Organisations & Initiatives:

- European Expert Group on Orphan Drug Incentives (OD Expert Group)
- EFPIA European Federation of Pharmaceutical Industries and Associations Patient Think Tank
- EUROPABIO Patients Advisory Group
- European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)
- EUPATI-Spain (partnering on EuroCAB)
- RARE DISEASE MOONSHOT – scaling up public private partnerships to accelerate researchin rare diseases
- Genomics England
- European Children's Hospitals Organisation (ECHO)

Partnership with Learned Societies:

- European Hospital & Healthcare Federation (HOPE)
- European Society of Human Genetics (ESHG)
- European Hematology Association Patient Advocacy Committee (EHA PAC)
- European Connected Health Alliance – ECHAlliance
- Rare Cancers Working Group of the European Society for Medical Oncology (ESMO)
- European Alliance for Personalised Medicine
- European Association of Health Law
- Society for the Study of Inborn Errors of Metabolism (SSIEM)

Member of European Projects:

RealiseD - Comprehensive methodological and operational approach to clinical trials in ultra-rare diseases

c4c - conect4children

ERDERA (European Rare Diseases Research Alliance and Partnership)

MoreEuropa - More Effectively Using Registries to support Patient-centered Regulatory and HTA decision-making

EUCAPA - European Capacity Building for Patients (end Feb 2025)

REMEDI4ALL - The European Platform for Medicines Repurposing

Screen4Care - Shortening the path to RD diagnosis by using newborn genetic screening and digital technologies trials

FACILITATE - Framework for Clinical Trial Participants' Data Reutilization for a Fully Transparent and Ethical Ecosystem

ERICA – European Rare Disease Research Coordination and Support Action

JARDIN - Joint Action on ERN integration into National Healthcare Systems

LIVES – Quality of life of patients living with vascular liver diseases. Developing research on the social impact of rare diseases

JOIN4ATMP - Map, join and drive European activities for Advanced Therapy Medicinal Product Development and implementation for the benefits of patients and society

International Institutions, Not-for-Profit Organisations & Initiatives:

RDI: Rare Diseases International (founding member)

WHO–Europe – Novel Medicine Platform

NGO Committee for Rare Diseases (United Nations, New York) (founding member)

IRDiRC: International Rare Disease Research Consortium (founding member)

Global Commission to end the diagnostic odyssey for children (founding member)

UDNI – Undiagnosed Diseases Network International

International partnerships (MoUs): NORD (USA), JPA (Japan)

IAPO: International Alliance of Patients' Organizations

iCONS: The International Consortium on Newborn Sequencing

PFMD - Patient Focused Medicines Development Initiative

ORPHANET

Partnering with European Initiatives

BBMRI Stakeholders Forum

HealthData@EU Pilot project - External Advisory Board

Together4 Rare Diseases

DeCODe- Medical devices - Supporting the development of paediatric and orphan devices

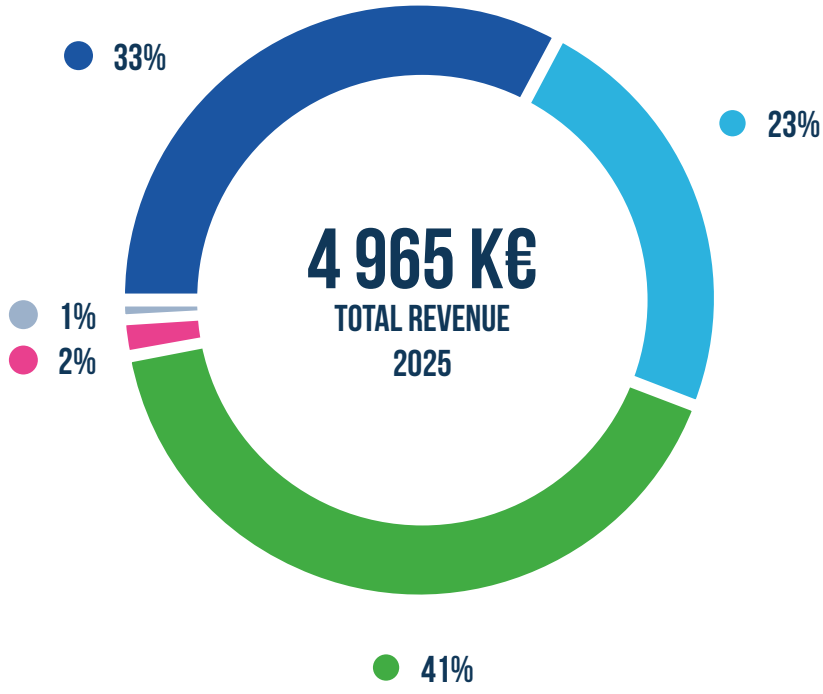
RWE4Decisions

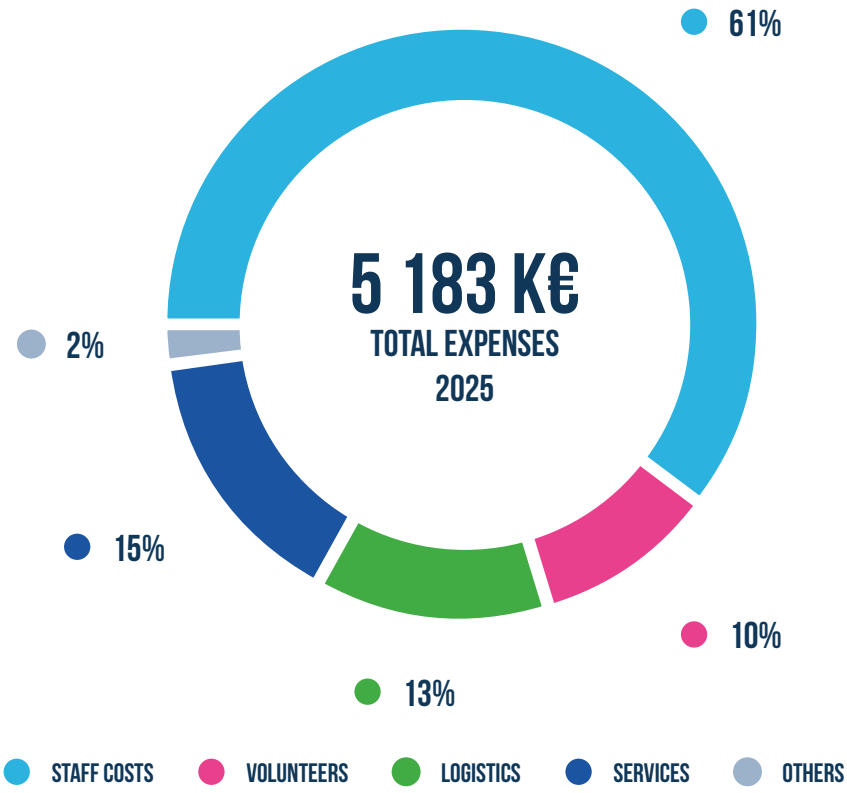
EuroCAB programme

SIMPATHIC Accelerating drug repurposing for rare neuro disorders by exploiting SIMilarities in clinical and molecular PATHology

EURAS - EUropean network for neurodevelopmental RASopathies

REVENUES & EXPENSES 2025





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RARE DISEASES EUROPE

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