EURORDIS
RARE DISEASES EUROPE

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WORK PROGRAMME 2024

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RARE DISEASES EUROPE

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COVER PHOTO: "RARE BEAUTY"
BRIANNA DOSE
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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 representing 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 ACHIEVE MEASURABLE GOALS PROLONGING AND IMPROVING THE LIVES OF PEOPLE LIVING WITH RARE DISEASES BY 2030

"NEVER STOP HAVING FUN"
ORNITHINE TRANSCARBAMYLASE DEFICIENCY, A UREA CYCLE DISORDER / USA

DELIVERING ON PRIORITY AREAS:

- DIAGNOSIS
- RESEARCH & DEVELOPMENT
- DATA & DIGITAL HEALTH
- INTEGRATED CARE
- HEALTHCARE
- TREATMENTS

"JACOB AND MICHAEL IN THE GARDEN"
ORNITHINE TRANSCARBAMYLASE DEFICIENCY, A UREA CYCLE DISORDER / USA

INCLUSIVE OF ALL RARE DISEASES, ALL REGIONS, "LEAVING NO ONE BEHIND" BY COVERING ALL THERAPEUTIC AREAS, ALL COUNTRIES IN GEOGRAPHICAL EUROPE, AND ALL RARE DISEASE PREVALENCE AND INCIDENCE LEVELS

"MY OTHER HALF"
PRIMARY SCLEROSING CHOLANGITIS / CANADA

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

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

ADVOCATE | EMPOWER | PARTNER

"THIS IS US LOVING RARE"
VANISHING WHITE MATTER DISEASE / USA

ADVOCATE

In 2024, we will explore and consolidate the EU's political options to reassess and update the EU's rare disease strategy.

This initiative will be guided by the Rare 2030 policy recommendations and will involve continued advocacy for a European Action Plan for Rare Diseases. Leveraging endorsements from prior Presidencies of the Council of the EU for an Action Plan, EURORDIS will collaborate with the Presidencies of 2024. This cooperation will lay the foundation for engaging with the European Parliament and the European Commission subsequent to the 2024 European Parliament elections.

We will safeguard initiatives impacting rare diseases within the EU multi-annual financial framework 2021-2027 and the EU4Health Programme 2021-2027.

This will include consolidating:

- The sustainability of the European Reference Networks;
- Support for pediatric and adult rare cancers under the EU Beating Cancer Plan;
- The European Rare Disease Research Alliance (ERDERA);
- New flagship initiatives, such as EU-level collaboration on newborn screening;
- EU funds and mechanisms for improving access to treatments for rare diseases.

We will engage the Network of National Alliances (Council of National Alliances - CNA) in advocacy efforts, using both in-person meetings and monthly 'hot topics' calls.

EURORDIS will equip the CNA with medium-term advocacy strategies to facilitate

forthcoming interactions, providing resources and information that empower the National Alliances to lead Member States' involvement in EU initiatives and legislation within key strategic areas. In 2024, EURORDIS will support the EU National Alliances in a collaborative EU-wide campaign based on the EURORDIS Manifesto, 'Championing the Rare: Building the Engine of an Inclusive European Health Union'.

Within and in collaboration with RDI, engaging the CNA and CEF, we will support the development of the key asks of the rare disease (RD) community in Europe, through expert collaboration to draft the WHO Resolution on Rare Diseases; cooperate with WHO Member States from the EU towards its adoption at the World Health Assembly (WHA) 2025.

The work towards the WHO Resolution is inspired by the results of policies in Europe, the Foresight study Rare2030, the national plans on RD. We will co-organise an international policy side event, with RDI and the NGO Committee for Rare Diseases, at the WHA in May 2024.

We will:

- Promote Europe's implementation of the United Nations' Political Declaration on Universal Health Coverage (2019) together with the UN General Assembly Resolution on Addressing the Challenges of Persons Living with a Rare Disease and their Families (2021), informed by the Rare 2030 recommendations;
- Promote the draft WHO Resolution on RDs, in order to cooperate with WHO / Europe on future actions on rare diseases with a focus on non-EU countries.

EMPOWER

BUILDING THE COMMUNITY, CAPACITY-BUILDING, INFORMING, SUPPORTING, AND EMPOWERING OUR NETWORKS

We will continue to develop and engage the network of over 1,000 EURORDIS Members, with a special focus in 2024-2025 on Eastern European, rare cancers, rare mental disorders, rare infectious diseases, and rare mitochondrial disease organisations, as well as those representing underrepresented therapeutic areas.

We will also reach out to all European patient organisations at large, develop the EURORDIS contact database, and interact with Members through the bi-monthly Member News.

We will coordinate the European Network of National Alliances for Rare Diseases by:

- Hosting a monthly online meeting on key issues, plus two face-to-face CNA meetings;

- Aligning the National Alliances with EURORDIS' strategy;
- Engaging National Alliances in advocacy through monthly newsletters, ongoing emails, and special group meetings;
- Fortifying the Network with frequent one-on-one meetings, peer training, and exchanges of best practices.

We will coordinate the European Network of Disease-Specific European Federations and provide continuous support to 20 of the weakest or youngest rare disease European Federations by co-financing their Network meetings and working more closely with them on key strategic items.

We will coordinate the European Network of Rare Disease Helplines (ENRDHL) by:

- Identifying common interests;
- Analysing the profile of callers and the purpose of their inquiries;
- Exploring the use of artificial intelligence in the processing of calls.

In addition, we will support the helplines to deal with reports from patients via the online EURORDIS Access Campaign form disseminated in 2023.

BUILDING CAPACITY FOR OUR NETWORKS

Following the 2024 European Parliament elections and upon the relaunch of the European Network of Parliamentary Advocates for Rare Diseases, we will organise Brussels Rare Diseases Week (RDW) 2024.

This event will host a hybrid advocacy and learning programme for patient advocates, focused on collective and specific policy asks. Facilitated interactions between RDW advocates and decision-makers, including newly elected Members of the European Parliament (MEPs), will foster dialogue between the groups.

We will host the Black Pearl Awards (BPA) 2024 in February 2024 as a hybrid event gathering up to 250 people in Brussels and up to 200 people online via an interactive platform. Participants will enjoy an awards ceremony, artistic performances, official Rare Disease Day animations, and keynote speeches.

PARTNER

We will organise the 12th European Conference on Rare Diseases and Orphan Products (ECRD) 2024 as a hybrid event under the auspices of the Belgian Presidency of the EU Council, gathering up to 300 people in Brussels on 15-16 May 2024 and up to 700 people online via an interactive platform.

300 PEOPLE	IN BRUSSELS 15-16 MAY 2024
UP TO 700 PEOPLE	ONLINE INTERACTIVE PLATFORM

The overarching title of the conference is 'Action Within Reach: Pioneering Solutions for Rare Diseases'. Sessions will cover the following topics:

- Diagnosis and screening;
- Research and innovation;
- The accessibility, availability and affordability of treatments;
- Mental wellbeing;
- Access to highly specialised healthcare;
- EU and national rare disease plans.

Discussions at ECRD 2024 will result in clear policy recommendations, shaping future EU and national policies.

STRATEGIC OBJECTIVE 02:

DELIVERING ON PRIORITY AREAS

EURORDIS will undertake a range of activities to achieve this Strategic Objective, in alignment with its overarching strategy to advocate, empower, and partner for people living with rare diseases.

These activities will fall within these key priority areas:

- HEALTHCARE POLICY AND SERVICES
- RESEARCH POLICY AND ACTIVITIES
- DATA AND DIGITAL HEALTH
- DEVELOPMENT AND ACCESS TO DIAGNOSTICS AND THERAPIES
- SOCIAL POLICY AND SERVICES

"HEALING"
HAPPLOINSUFFICIENCY MEF2C / POLAND

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

JARDIN KICK-OFF MEETING, MARCH 2024, BRUSSELS

ADVOCATE

We will strive to reduce diagnostic delays for rare diseases and tackle undiagnosed diseases by calling for a harmonised European approach to newborn screening based on our 11 Key Principles.

We will also continue to call for the formation of an EU-level expert working group to develop this approach and facilitate collaborative action among Member States.

We will build on the outcomes of the SOLVE-RD project and its 'Time to Act' appeal to EU institutions, advocating for the creation of an EU-wide reanalysis platform for undiagnosed cases.

This initiative will be closely coordinated with the Joint Action on Integration of ERNs into National Health Systems (JARDIN).

We will harness insights from the EURORDIS Rare Barometer programme, which captures the needs and experiences of people living with rare diseases, to shape our policy and research contributions.

We will focus on broadly disseminating the results of the 'Journey to Diagnosis' survey for people living with a rare disease, which garnered over 13,300 respondents globally and nearly 10,500 in Europe. We will ensure the results are presented in a peer-reviewed publication. We will also share findings from the 2023 survey on the opinions of people living with a rare disease and their families about newborn screening programmes.

RESPONDENTS

13,300
GLOBALLY

10,500
IN EUROPE

The Rare Barometer programme will design and launch a new survey on Mental Health and Wellbeing and will harness the results in the second part of 2024.

People living with rare diseases in the EU living at the intersection of multiple groups in vulnerable situations that increased vulnerability and risk to mental health risk factors and determinants. The survey will help understanding and evidencing the specificities and the unmet needs of PLWRD regarding mental health and wellbeing. The results will be widely shared and disseminated to the EURORDIS Mental Health and Wellbeing Partnership Network, through EURORDIS' membership and communication channels.

We will champion enhanced European collaboration to facilitate access to cross-border, highly specialised healthcare for rare and ultra-rare conditions.

This effort will include developing with our members and stakeholders a European model for service planning, contracting, and delivery, and shaping care pathways.

We will seek to secure political backing and financial stability for the ERNs, while also identifying improvement areas and devising solutions for the shortcomings highlighted in the Networks' five-year evaluation.

EMPOWER

We will continue the EURORDIS Newborn Screening Working Group (NBS-WG) and our efforts to promote our 11 Key Principles for newborn screening.

In 2024, we will maintain engagement with EU Institutions to create new, EU-funded initiatives or projects addressing EU-wide disparities in this area. Additionally, we will aim to join the International Consortium on Newborn Sequencing, which is centralising global newborn genomics research.

We will support the implementation of the Patient Partnership Framework in European Reference Networks (ERNs), in collaboration with ePAG advocates and ERN coordinators, through:

- Identifying and bridging gaps by co-developing tools and resources for effective ERN partnerships;
- Facilitating peer learning and best practice exchange in ePAG advocate transversal working groups;

- Advising ERNs on engaging patient representatives in key initiatives, such as clinical guideline development;
- Hosting an annual ePAG Steering Committee meeting in Barcelona to review progress and share successful strategies.

Through engaging in 2024 with the ePAG advocates and ERN project managers, we will implement the first stage of a knowledge management strategy to:

- Develop a taxonomy and online repository of tools and resources for patient partnership in the ERNs to facilitate the use and reuse of these assets;
- Categorise existing resources, starting with the tools and resources developed by EURORDIS. In a second stage, content developed by other organisations will be added;
- Create and implement an online repository.

We will support the National Alliances on ERN-related matters and engage with them on the activities of the Joint Action on Integration of ERNs into National Health Systems (JARDIN).

PARTNER

In collaboration with leading ERNs, we will guide the H-CARE project in 2024, focusing on dissemination of the 2023 literature review findings and organising focus groups for the second phase of PREM validation.

We will participate in the new Joint Action on Integration of European Reference Networks into National Health Systems (JARDIN) (January 2024-2027, DG Sante). This participation will involve:

- Collaborating with and assisting patient organisations engaged in the various activities of the Joint Action;
- Fulfilling EURORDIS responsibilities in the Joint Action, focusing on undiagnosed programmes, national expertise centre networks, model care pathways, case management guides, crisis readiness, and health data interoperability.

We will maintain our institutional relationship with the ERN coordinators, hospital managers, and Board of Member States representatives to contribute to the strategic planning of the ERNs for the next five-year cycle.

"EXPLORE MORE"
ORNITHINE TRANSCARBAMYLASE DEFICIENCY, A UREA CYCLE DISORDER / USA

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

Will continue the contribution to the RD Moonshot[1] to enhance public-private partnerships, in particular by disseminating and advocating for the operationalisation of the RD Moonshot diagnostic research recommendations co-drafted by EURORDIS in 2023.

[1] The RD Moonshot is a coalition of partners from industry, research and patient organisations joining forces to accelerate scientific discovery and drug development in rare and paediatric diseases for which currently there is no therapeutic option.

EMPOWER

We will develop the Open Academy e-learning courses and digital platform by continuing to develop and improve the delivery of the pre-training for both the School on Medicines Research and Development and the School on Scientific Innovation and Translational Research. We will also develop an online introductory course on genetics and genomics.

We will improve outreach to the network of Open Academy alumni through monthly newsletters, online webinars, and structured communication.

This will help facilitate their transition from trainees to active alumni, including follow-up training, post-school webinars, and online alumni masterclasses using a continuing education approach and peer-to-peer exchange.

PARTNER

TRANSLATIONAL RESEARCH

We will support the establishment of ERDERA (European Rare Diseases Research Alliance and Partnership, September 2024-August 2031, Horizon Europe)[2] to ensure that the needs of people living with rare diseases are reflected.

Since November 2021, EURORDIS has been fully engaged in co-designing ERDERA together with the main European rare disease research stakeholders. EURORDIS has co-developed the ERDERA Strategic Research and Innovative Agenda (SRIA) as well as the project proposal submitted to the European Commission in September 2023. ERDERA will start in September 2024, leveraging the European Joint Project on Rare Diseases outcomes.

We will continue participating in the European Joint Programme on Rare Diseases (EJP-RD, January 2019 – September 2024, Horizon 2020, Partner). This participation will involve:

[2] Rare Disease Partnership, a major research programme meant to start by late 2024.

- Continuing to co-lead the EJP RD Pillar 3 Activities on training and capacity building;
- Continuing involvement in the dissemination, mentoring and management of the three EJP RD Massive Open Online Courses (MOOCs) co-developed by EURORDIS on diagnosis, translational research and rare disease data;
- Delivering face-to-face trainings within the Open Academy Schools on Medicines Research & Development and on Scientific Innovation & Translational Research;
- Further surveying patients' perceptions of their involvement in current and future research projects funded by EJP RD;
- Engaging patients in evaluation workshops;
- Contributing to updates to, and the development of, contemporary or new content for the Orphan Drug Development Guidebook.

We will continue participation in the European Rare Disease Research Coordination and Support Action (ERICA, March 2021 – February 2025, Horizon Europe, Partner) project up to February 2025.

Toward this end, we will set up a Task Force composed of ePAG advocates and clinicians involved in the ERNs to scope and co-develop a patient partnership framework for clinical trials.

UNDIAGNOSED DISEASES

We will continue to represent EU rare disease patients' perspectives and the priority of the undiagnosed community by:

- Participating in the Undiagnosed Diseases Network International (UDNI);
- Coordinating the UDNI Patient Engagement Working Group in partnership with the National Organization for Rare Disorders (NORD) and the Wilhelm Foundation;
- Coordinating activities between the Joint Action on Integration of ERNs into National Health Systems (JARDIN) work package on national reference networks and undiagnosed disease programmes, or coordinating equivalent strategies linking ERN and UDNI actions within the EU.

"GROW UP WITH LOVE"
OCULOCUTANEOUS ALBINISM / TURKEY

PAEDIATRIC CLINICAL TRIALS

We will continue to participate in Conect4Children (C4C, May 2018 - April 2024, extended until April 2025, IMI 2, Partner) by:

- Contributing to the external stakeholder management process of the pan-European Paediatric Clinical Research Network and the sustainability plan of this network;
- Bringing to the project EURORDIS expertise on patient engagement;
- Training patients and disseminating the work of Conect4Children in webinars and conferences;
- Contributing to the work on data quality standards and to the project's Education and Training programme.

PATIENT ENGAGEMENT IN RESEARCH

We will continue to advocate for the CABs and work with partners to ensure there are credible, patient-led community advisory boards available.

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.

PARTICIPANTS AT THE EURORDIS OPEN ACADEMY 2023

ADVOCATE

We will advocate for a European Health Data Space (EHDS) to enable the secure sharing and use of data at the EU level in the context of the legislative proposal debated in 2024 by the EU Institutions.

We will promote the construction of an EHDS that optimises electronic health records, ensures the ethical use of data for research, policymaking, and treatment development, increases digital health literacy in the rare disease community and beyond, and encourages the formation of patient and public partnerships.

EMPOWER

We will promote digital health literacy, including through training on consent.

This training will teach participants to understand the process of obtaining and withdrawing consent, identify differences between consent for primary and secondary health data uses, explore other safeguards beyond consent, and identify situations in which consent may not be asked.

We will coordinate the EURORDIS Digital Advisory Group (DAG), providing capacity-building sessions for DAG's members to be well informed and prepared to deliver their opinion on matters relating to digital health.

PARTNER

We will participate in the EU project FACILITATE[3] (January 2022-December 2025, IMI2, Partner), which is investigating ways to return data to patients participating in clinical trials. In 2024, EURORDIS will contribute to:

- The return and reuse of clinical data ethical frameworks and data workflows;
- The project's legal framework and the glossary of terms;
- The project's platform specifications and requirements;
- A literature review on patients' willingness to share their clinical trial data;
- Expanding the project's visibility by creating a Patient Engagement Open Forum;
- Bridging the project's connection with the European Medicines Agency (EMA).

We will continue to be an active member of the Together4RD[4] initiative supporting ERNs and industry collaboration.

EURORDIS will support the development of several pilots of ERN collaboration with industry, supporting patient involvement in the design and development of the pilots. In addition, we will support the development of an ERN toolkit with approaches, tools and processes that can support ERN-industry partnerships based on existing collaborations. Finally, EURORDIS will engage with the patient community to advocate for a revision to the Board of Member States' Statement on 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!

[3] **FACILITATE's** main objective is the development of a new ethical, legal, and regulatory framework to enable the return of clinical trial data to study participants and the healthcare professionals involved in their care. The secondary objective is to build a prototype process enabling the reuse of that data for future research needs.

[4] **Together4RD** is a multi-stakeholder initiative aimed at supporting collaboration between European Reference Networks (ERNs) and industry in areas that will address the unmet medical needs related to the 95% of rare diseases without a dedicated treatment.

ADVOCATE

We will advocate for the implementation of an EU pharmaceutical regulatory and policy framework that addresses the challenges of people living with rare diseases.

This will primarily involve contributing to the revision of the general pharmaceutical legislation, which includes revisions to the Regulation on Orphan Medicinal Products and Paediatric Medicines. To influence these revisions, we will:

- Continue calling on MEPs to improve the legislation within the ongoing legislative process;
- Support National Alliances with outreach to individual Member State governments and contribute to the work of the Belgian and Hungarian Presidencies of the EU Council;
- Integrate discussions on the general pharmaceutical legislation into upcoming events, such as EURORDIS Roundtable of Companies (ERTC) meetings and ECRD 2024;
- Educate new MEPs about the legislative revisions following elections in May 2024.

We will advocate for the strengthening of EU clinical research capabilities in the context of the ACT-EU initiative.

We will formulate new advocacy messages on drug repurposing, using the results from our work on the DITA Task Force, STAMP[5] and REMEDi4ALL[6].

We will advocate for the implementation of the Regulation on EU Cooperation on Health Technology Assessment by contributing to the consultations on implementing acts and to meetings of the EU Health Technology Assessment (HTA) Cooperation Stakeholder Network.

We will also follow European and national discussions on the implementation of the Regulation from 2025, and continue the dialogue on pricing policies and methodological guidelines for the economic evaluation of health technologies. The EUCAPA project to train patient advocates on HTA will accompany these activities.

We will advocate for improved access to treatments for rare disease patients by promoting the proposal of structured cooperation across European countries in pricing and reimbursement policies and the development of funding mechanisms.

[5] **The STAMP** project connects academic researchers with medicine regulatory agencies for early scientific advice on repurposing projects. This is instrumental in generating a robust data package to translate research into access to medicines for patients.

[6] **REMEDi4ALL** aims to establish Europe's leadership globally in the repurposing of medicines by creating a vibrant community of practice covering all relevant sectors and disciplines.

We will also engage with the WHO/Europe Novel Medicines Platform (NMP), which was launched in September 2023 for 3 years up to June 2026. EURORDIS is engaged as a full partner with a Non-State Actor Status and is a member of the WG Solidarity (Vice Chair) and the WG Sustainability. A focus is on improving access, solidarity and sustainability to very low prevalence RDs, highly complex treatments and highly expensive ones.

We will call for the harmonisation of Compassionate Use Programmes (CUP) and a European fund for CUP.

We will continue work on the International Council for Harmonisation (ICH) Principles for Clinical Trials in 2024, contributing to the annexes on traditional clinical trials and innovative trials.

EMPOWER

We will deliver two Schools in 2024 on 'Medicines Research & Development' and 'Scientific Innovation & Translational Research' to empower patient representatives with the confidence and knowledge needed to bring expertise to discussions on healthcare, research, and medicines development. The two schools will gather around 70 participants in Barcelona in June.

We will support patient engagement in EMA activities via EURORDIS' representation on the EMA Management Board and the body's various committees, including the Patients' and Consumers' Working Party (PCWP). We will also:

- Support patient representatives on the Committee for Orphan Medicinal Products (COMP), the Paediatric Committee (PDCO), the Committee for Advanced Therapies (CAT), and the Pharmacovigilance and Risk Assessment Committee (PRAC);
- Involve and support patient representatives within the working groups of the different EMA Scientific Committees;
- Facilitate the EURORDIS Therapies Action Group (TAG), which gathers EURORDIS volunteers participating in the EMA Committees;
- Identify experts for Scientific Advisory Group meetings, disseminate EMA Scientific Committee Surveys to relevant rare disease groups, and collaborate with the EMA to identify, support and mentor patient experts in Scientific Advice and Protocol Assistance procedures;
- Identify experts for Scientific Advisory Group meetings, disseminate EMA Scientific Committee Surveys to relevant rare disease groups, and collaborate with the EMA to identify, support and mentor patient experts in Scientific Advice and Protocol Assistance procedures;
- Develop and disseminate monthly Therapeutic Reports.

We will support patient involvement in HTA activities through the coordination of the EURORDIS HTA Task Force (HTA TF).

We will also contribute to the implementation of the EU HTA Regulation (2021/2282), and provide guidance on methods to involve patients in horizon scanning, joint scientific consultations, coping, and joint scientific assessments. The HTA TF will also follow discussions on newly authorised medicines at the national level and discussions on access and reimbursement.

We will enhance the capabilities and expertise of patient advocates in Health Technology Assessments (HTAs) through the EUCAPA – HTA training (EU4Health, March 2023-March 2025, Coordinator), guaranteeing patient perspectives are integral from the outset of EU HTA cooperation.

EURORDIS spearheads this project, crafting the training content for the introductory course and overseeing patient recruitment and selection.

EURORDIS spearheads this project, crafting the training content for the introductory course and overseeing patient recruitment and selection.

Additionally, we will distribute the peer-reviewed paper detailing the 10 years of insights from early and multi-stakeholder dialogues through MoCA[7].

[7] Cavaller-Bellaubi, M., Hughes-Wilson, W., Kubinová, Š. et al. Patients, payers and developers of Orphan Medicinal Products: lessons learned from 10 years' multi-stakeholder dialogue on improving access in Europe via MoCA. Orphanet J Rare Dis 18, 144 (2023). https://doi.org/10.1186/s13023-023-02774-7

We will support patient involvement in developing quality information on medicines, through the coordination of the EURORDIS Drug Information, Transparency and Access Task Force (DITA TF).

This will involve reflecting on how our members can be better informed about when they need to prepare for the evaluation of medicines and health technologies, and how to work in parallel with the Patients' and Consumers' Working Party (PCWP).

PARTNER

We will back the initiation of the new EU project JOIN4ATMP (January 2024 – December 2026, Horizon Europe), aimed at streamlining the progression of advanced therapy medicinal products (ATMPs) from preclinical to clinical stages.

This project will pinpoint existing barriers, devise solutions from real-world scenarios, and offer recommendations to expedite and reduce risks in European ATMP development ensuring broader ATMP accessibility. As a central collaborator, EURORDIS will engage in launching JOIN4ATMP, contribute to all work packages, and participate in the steering committee to support the project's coordination, communication, and dissemination.

We will participate in the EU project 'More Effectively Using Registries to support Patient-centred Regulatory and HTA decision-making' (More-EUROPA, 2023-2028, Horizon Europe), which aims to develop, implement and establish standards and methods to address the data and evidentiary needs of regulatory authorities and HTA bodies.

The end goal is to progress the more efficient use of Real- World Evidence (RWE) for the development, registration and assessment of medicinal products in Europe. In 2024, together with the European Multiple Sclerosis Platform, EURORDIS will develop training content for patient advocates. EURORDIS will also create a group of volunteers involved in the governance of registries to interact with More-EUROPA researchers.

We will continue to participate in the EU project Next Generation Health Technology Assessment (HTx[8], January 2019-June 2024, Horizon 2020, Partner) by:

- Leading the development of training materials for patients and project participants, and disseminating project updates and information.
- Conducting a study with patients and healthcare professionals to investigate their receptiveness to incorporating treatment costs into shared decision-making processes. This will also include finalising a toolkit that encapsulates key project findings through videos, recordings, and written materials.

[8] **HTx** aims to create a framework for the Next Generation Health Technology Assessment to support patient-centred, societally oriented, real-time decision-making on access to and reimbursement for health technologies throughout Europe.

We will participate in the GetReal Institute[9] as the co-founder and Board member working on reducing barriers to the use of secondary data sources, and address the evidence needs of downstream healthcare decision-makers.

In 2024, the second GetReal Institute annual conference will be organised with the participation of EURORDIS. Several working groups will be created on topics such as Collaboration & Engagement, Data & Methods, Knowledge Dissemination, and Education & Training.

We will participate in the EU project VACCELERATE[10] (January 2021-January 2024, Horizon 2020, Advisory) which establishes a platform linking European vaccine development stakeholders, mapping clinical trial and laboratory sites to identify optimal locations for Phase 2 and 3 vaccine trials across Europe.

VACCELERATE will leverage its experience and expertise in adult and paediatric master protocols to design and implement an Adaptive Platform Trial (VACCELERATE APT).

"YOU FILL UP MY SENSES"
MICROPHTHALMIA SYNDROME 12 (MCOPS12)
USA

We will work to create more treatments for rare conditions via drug repurposing (REMEDi4ALL[11], September 2022-August 2027, Horizon Europe, Partner).

Toward this end, we will actively work with all stakeholders to ensure meaningful patient engagement, patient partnership and co-creation throughout the entire drug development process and collaborate in the development of training materials for patients and all relevant stakeholders.

We will continue promoting the patient voice by chairing the Screen4Care (S4C, September 2021-September 2026, IMI 2, Partner)[12] Patient Advisory Board (PAB), which consists of 15 patient representatives involved in the EURORDIS Newborn Screening Working Group (NBS-WG), the Digital and Data Advisory Group (DAG), and ePAGs.

The PAB provides strategic recommendations, guidance, and advice across all Screen4Care activities. We will finalise the recommendations on actionable rare diseases, guided by

[9] **GetReal Institute** builds on the success of two IMI projects: GetReal and The GetReal Initiative and brings together a wide variety of stakeholders to drive the sustainable development and adoption of tools, methods and best practices in the generation and use of RWE (Real-World Evidence) for better healthcare decision-making.

[10] **VACCELERATE** is a clinical research network for the coordination and conduct of COVID-19 vaccine trials. The network is comprised of academic institutions from all over Europe: The consortium is led by the University Hospital Cologne, Germany, and currently includes 29 national partners in 18 EU-member states and five countries associated to the EU Horizon 2020 research programme.

[11] **REMEDi4ALL** aims to build a sustainable European Innovation Platform to enhance the repurposing of medicines. The project is expected to make a major leap forward in patient-centric drug repurposing, or finding new therapeutic options for existing drugs, in areas where there are high unmet medical needs, including the rare disease field.

[12] The aim of Screen4Care is to accelerate the diagnosis of rare diseases based on two central pillars: genetic newborn screenin and digital technologies.

stakeholder engagement. Our ongoing engagement with the Newborn Screening Forum, through both online and in-person meetings, will focus on discussing genetic newborn screening and ensuring that S4C results are thoroughly analysed and shared with the rare disease patient community and key stakeholders.

We will continue representing patients in the IRDiRC (International Rare Diseases Research Consortium) General Assembly and the Patient Advocacy Constituent Committee (PACC), as well as in the Consortium's Therapies Scientific Committee (TSC). Specifically, EURORDIS will:

- Finalise the work done by the Task Force on Repurposing and Disregarded Diseases (PLUTO project) which aims to use an integrated database search approach to identify and classify the groups of rare diseases that are currently under-represented by academic research and industrial development;
- Contribute to the Task Force on Functional Analysis for the creation of a framework for the robust and effective ecosystem of functional analyses in rare diseases;
- Contribute to the Taskforce on Preparing for genetic N-of-1 treatments of patients with ultra-rare mutations;
- Contribute to IRDiRC activities related to newborn screening in synergy with EURORDIS activities;
- Follow-up on the work carried out within IRDiRC on the Clinical Research Networks for Rare Diseases;
- Participate in the IRDiRC-RDI Global Access Working Group, linking this Group to the Novel Medicines Platform of WHO Europe.

We will actively engage in the working group of the Global Commission to End the Diagnostic Odyssey for Children with Rare Diseases, as the initiative was being reignited in autumn 2023.

We will actively contribute to the shaping and development of the Global Commission in its three areas of activities: patients and family empowerment, equipping health providers, and policy.

"TOGETHER STRONG"
PELIZAEUS-MERZBACHER DISEASE / AUSTRIA

SOCIAL POLICY AND SERVICES 05[05]

> 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

We will advocate for integrated and holistic care as a priority within the European Action Plan for Rare Diseases.

We will also support ERNs to advance case management for people living with rare diseases, through the Joint Action on ERN Integration into National Health Systems (JARDIN).

We will call for the rare disease community's access to quality and adequate social and employment rights by contributing to consultations on EU initiatives and legislative

proposals resulting from the EU Pillar of Social Rights Action Plan, in cooperation with the Social Platform.

We will support improved access for people living with rare diseases to disability rights and independent living support, as an observer member of the EU Disability Platform and member of the European Disability Forum.

We will continue to contribute to EU consultations and discussions on the various flagship initiatives of the European Strategy for the Rights of Persons with Disabilities 2021-2030, including direct advocacy to influence the legislative proposal for a Directive establishing the European Disability Card and the European Parking Card.

The Rare Barometer programme will conduct a new survey looking into the disabilities and independent living daily barriers experienced by people with rare diseases, the recognition of their disabilities, and their access to disability rights.

We will advocate for the new EU Comprehensive Approach to Mental Health to be inclusive of all people in vulnerable situations, specifically people with an existing health condition, such as chronic conditions and rare diseases, with the support and engagement of the new EURORDIS Mental Health & Wellbeing Partnership Network. A position paper on mental health and wellbeing will be adopted in early 2024.

We will support the establishment of the EURORDIS Mental Health & Wellbeing Partnership Network, by implementing the governance structure, operational procedures of the Network, and supporting the members to be active in steering and working groups.

We will promote the UN General Assembly Resolution call for governments to develop psychosocial programmes for rare diseases.

EMPOWER

We will initiate the activities of the newly formed EURORDIS Social Policy Action Group (SPAG), recruited in November 2023.

The SPAG supports EURORDIS in shaping policies and practices that improve holistic care access for people with rare diseases and their families, while also ensuring the protection of their social and human rights.

In 2024, the EURORDIS Mental Health & Wellbeing Partnership Network will facilitate connections between affected individuals and groups, specifically to identify needs, raise awareness, and advocate for access to self-management tools and psychosocial care.

The Network will start a co-creation process to develop a Mentally Healthy Toolkit, with evidence-based tools that can be tailored to the needs of different groups in vulnerable situations. The Network will also perform a gap analysis to evaluate current best practices and evidence, and initiate a series of literature reviews. These reviews will aim to strengthen the evidence base for identifying and addressing prevalent symptoms related to mental health issues.

PARTNER

We will engage with the European Commission to provide input to the design of future funding instruments, building on EURORDIS' extensive involvement in the review of the Employment and Social Innovation (EaSI) programme.

We will identify and seize opportunities to develop project proposals aimed at addressing key holistic and social care priorities, notably within relevant EU funding instruments.

We will continue to seek new opportunities to scale up the INNOVCare project, supporting the implementation of further case management services and resource centre services.

"GET BREATHLESS FOR PULMONARY HYPERTENSION UNDER THE SEA"
PULOMARY HYPERTENSION / CROATIA

STRATEGIC OBJECTIVE 03:

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

"MY LITTLE FOX"
FOX P1 / LATVIA

DOUBLE WAR - UKRAINE'S EUROPEAN APPROACH TO ENSURING AND SUPPORTING PATIENTS WITH RARE DISEASES IN THE CONTEXT OF FULL-SCALE WAR
KYIV, UKRAINE, 2024

ADVOCATE

We will continue to increase the visibility of rare cancers, including them across our 2024-2025 advocacy activities.

In addition to the work performed in ERNs, the Rare Cancer Advocates Network has identified priority advocacy topics to collectively work on:

- Contributing to the revision of the EU pharmaceutical legislation;
- Integrating rare adult cancers and paediatric cancers into national cancer plans;
- Fostering innovation beyond medicines;
- Contributing to the revision of the EU pharmaceutical legislation;
- Working on business models for ultra-rare cancers.

We will continue to work on policy initiatives and solutions tailored for the rarest diseases, according to the prevalence.

Our approach will include advocacy efforts to revise the EU Regulation on Orphan 15 Medicinal Products, contributing to projects like JOIN4ATMP and Screen4Care, engaging with EMA Committees, advocating for access to advanced therapies for very rare diseases, and promoting the delivery of specialised services through the ERNs at the EU level.

With a focus on non-EU countries of Eastern Europe and the Balkans, we will conduct research interviews and publish country-specific assessments on the state of play of rare diseases from the perspective of the patient communities. We will also compile a summary of recommendations for EURORDIS to better enfranchise the region in our work.

EMPOWER AND PARTNER

We will coordinate the Rare Cancer Advocates Network, launched in January 2023 and made up of 35 rare cancer ePAG advocates, united in carrying out collective advocacy actions to improve research and care for rare cancers, and joining forces on transversal issues relevant to all rare disease patients and caregivers.

EURORDIS has been uniting since February 2022 for the two million Ukrainians living with a rare disease within and outside of Ukraine, and we will continue our support in 2024 by:

- Supporting the preparation and participation of the Ukrainian Hub and Centres of Expertise in the JARDIN Joint Action starting in March 2024;
- Completing research on rare disease activities and patient organisation engagement in Eastern and South-Eastern Europe;
- Preparing to integrate support for Ukrainian patient groups into our regular operations, with the completion of the Ukraine Response Programme during the first quarter of 2024.

PARTNER

Through the European Rare Diseases Partnership and the European Rare Diseases Research Alliance (ERDERA), we will work to include under-represented countries into research networks.

RARE DISEASE DAY AWARENESS CAMPAIGN

The Rare Disease Day campaign will raise awareness at the European and international levels to achieve equity in social opportunities, mental and physical healthcare, and access to diagnosis and therapies for people living with rare diseases. The Rare Disease Day (RDD) campaign will:

- Coordinate the activities of RDD 2024, as well as prepare for RDD 2025. Both the 2024 and 2025 campaigns will be active throughout the year, with social media and patient stories in native languages incorporated into their communications.
- Focus on young advocates and healthcare providers;
- Co-create and produce RDD campaign materials based on strategic review recommendations and a call to action;
- Translate media assets and webinars to reach more and under-represented countries;
- Expand the 'Global Chain of Lights', while considering energy constraints and climate change;
- Improve the accessibility of RDD campaign materials, as well as the campaign's media coverage and outreach.

CROSS-CUTTING PRIORITIES

TO SUPPORT OUR STRATEGIC OBJECTIVES

EURORDIS STAFF, 2023

COMMUNICATION AND DISSEMINATION

01 04

We will evaluate, refresh and execute the new Communication and Dissemination Strategy in alignment with the overarching EURORDIS strategy by:

- Analysing current performance and finding innovative solutions to ensure the new EURORDIS website and digital outputs embody strategic objectives and adhere to accessibility standards;
- Facilitating the effective leveraging of our diverse networks to enhance participation in EURORDIS activities, spanning events and advocacy campaigns, and fostering synergies to accomplish strategic goals;
- Enhancing engagement with EURORDIS audiences on social media, newsletters, and podcasts by tailoring content to their interests, incorporating interactive videos, animations, and infographics where applicable;
- Ensuring uniform communication among EURORDIS staff, Board members, and volunteers through a shared brand identity, position papers, and talking points.

We will pioneer innovations in 2024 by:

- Giving greater attention to ultra-rare disease groups, young patient advocates, and members in Eastern and South-Eastern Europe on social media and in the EURORDIS website's Newsroom;
- Addressing the accessibility of our website and communication outputs;
- Elevating the recognition of volunteers through guest blogs, podcast episodes, interviews, and dedicated sections on the EURORDIS website.

PEOPLE - STAFF AND VOLUNTEERS

02 04

We will develop a People Management System to align HR function with strategic objectives and effectively manage staff and volunteers to support the organisation's mission by:

Enhancing team organisation that supports EURORDIS' strategy and operational needs with the appointment in 2024 of permanent positions, including the positions of:

- Human Resources Director (based in Paris, new, as of January 2024)
- Chief Executive Officer (based in Paris, succession, as of March 2024)
- Governance Senior Manager (based in Paris, succession, as of March 2024)
- Junior Events Manager (based in Paris, succession, as of March 2024)
- Budget and Compliance Manager (based in Paris, new, as of April 2024)
- Public Affairs Director (based in Brussels, succession, as of April 2024)
- Office Assistant (based in Paris, succession, as of April 2024)
- Open Academy Training Manager (based in Barcelona, succession, as of May 2024)
- Public Affairs Manager (based in Brussels, new, as of July 2024)

We will support a successful CEO transition to ensure sustainable leadership.

We will align our human resources to the new strategy by a targeted talent acquisition strategy.

We will provide ongoing training and development programs to enhance existing skills such as capacity building and communication skills, create career development and advancement opportunities to retain employees.

We will prioritise employee well-being and work-life balance by implementing policies and practices that promote a healthy and supportive work environment.

We will support the development of a 360-degree view of the organisation for a part of the management team to enhance transversal work strategically, streamline operations, and optimise time and fund allocation.

We will regularly assess and address the needs and expectations of employees and volunteers to ensure their satisfaction and engagement.

GOVERNANCE 03 04

We will distribute the revised EURORDIS strategy for 2021-2030, focusing on strategic alignment, improved organisational capabilities, and new management systems to achieve EURORDIS' strategic goals.

We will advance and strengthen EURORDIS' key partnership with its co-founder and primary supporter, AFM-Téléthon, under the renewed Memorandum of Understanding 2024-2027, facilitating regular interactions and collaborative efforts with the AFM-Téléthon ecosystem.

We will work toward a multi-annual budget for 2025-2027 that implements our strategy and better manages uncertainties with funding sources (EU4Health Operating Grant).

We will review and update EURORDIS by-laws, including the EURORDIS Policy on Financial Support by Commercial Companies, EURORDIS Guidelines & Protocol for Bilateral Meetings with Healthcare Companies, EURORDIS Policy for Declaration of Interests and Confidentiality Agreements.

We will review the strategic partnerships with international patient organisations and other stakeholders within the new international ecosystem of rare diseases.

RESOURCE DEVELOPMENT & EURORDIS SUSTAINABILITY 04

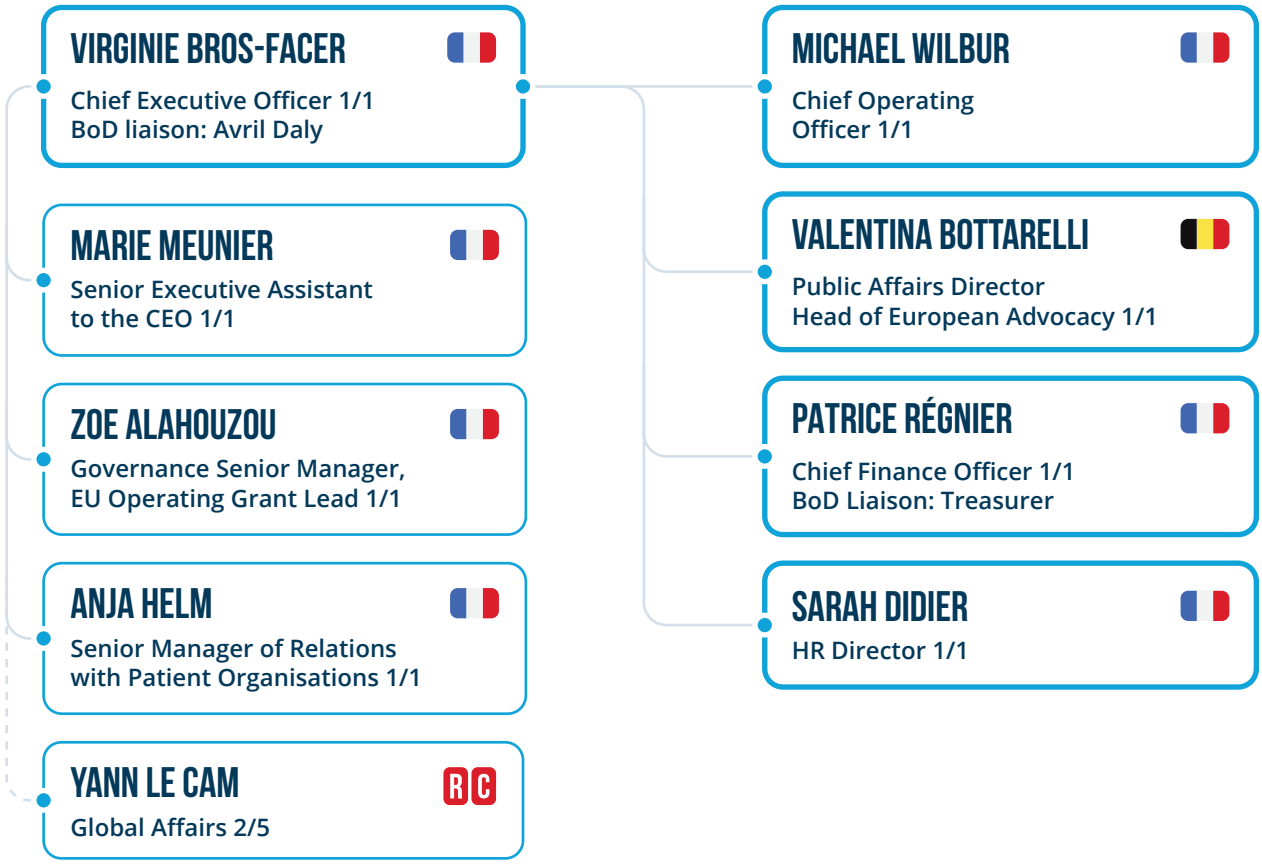
We will support the EURORDIS Round Table of Companies (ERTC) by engaging over 70 health companies in both bilateral and collective dialogue through regular webinars and two major face-to-face workshops focused on specific topics.

We will maintain activity to support current contributions from the health sector within the confines of the EURORDIS Policy on Financial Support from Commercial Companies.

We will seek new sources of funding to implement our strategic objectives, including diversified sources from foundations and individual donors.

We will invest in developing strategic projects based on innovative partnerships.

TEAM ORGANISATIONAL CHART 2024



PARIS OFFICE | BARCELONA OFFICE | BRUSSELS OFFICE | R WORK REMOTELY | T TEMPORARY | C CONTRACTORS


PATRICE RÉGNIER
Chief Finance Officer 1/1
BoD Liaison: Treasurer
SUSAN FOSTER
HR Support and Budget Manager 1/1
ANNIE RAHAJARIZAFY
Accounting Manager 1/1
TBR
Compliance & Manager 1/1
SARAH DIDIER
HR Director 1/1
CORINA PULS
Office Manager & IT Support 1/1
CLEMENT LE CHENE
Office Manager & People Team Support 1/1
VALENTINA BOTTARELLI
Public Affairs Director
Head of European & International Advocacy 1/1
ARIANE WEINMAN
Public Affairs Senior Manager – Rare Cancers 1/1
RAQUEL CASTRO
Social Policy & Initiatives Director 1/1
JULIE PERNET
EU Public Affairs Senior Manager 1/1
RACHEL BUTCHER
Public Affairs Manager 1/1 (UK)
From 01/07/2024
SOLÈNE JOUAN
EU Public Affairs Manager 1/1
ELIAS BARTH
Brussels Office Manager & Team Assistant 1/2
TBR
Public Affairs Director 1/1

TEAM ORGANISATIONAL CHART 2024

MICHAEL WILBUR
Chief Operating Officer 1/1

RAHUL WASLEKAR
Operations Junior Manager 1/1

FRANÇOIS HOUŸEZ
Information & Access to Therapies Director & Health Policy Advisor 1/1

JULIEN DELAYE
Patient Engagement Manager HTA 1/1

ROSELINE FAVRESSE
Research Policy & Initiatives Director 1/1

GULCIN GUMUS
Research & Policy Senior Manager 1/1

BRIAN HOWARD
Philanthropy and Partnerships Director 1/1

STANISLAV OSTAPENKO
Communications Director 1/1

JULIEN POULAIN
Communications Manager 1/1

ANDREA OSVOLL* (MAT LEAVE)
Communications Manager - Programmes & Projects 1/1

IMÈNE EL AINI
Web Technology Junior Manager 1/1

RHIANNON WALLS
Communications Junior Manager 1/1

LISE PERNIN
Communications Junior Manager – RDD 1/1

JELENA MALININA
Data Director 1/1

VERONICA POPA
Patient Engagement Manager: Digital & Data 3/5 (Bucharest)

* Replacement for Mat. Leave until Sept.24: Jessica BAILLOUX - Communications Junior Manager

** Replacement for Mat. Leave until Sept.24: Cindy PENNINGNIEUWLAND Corporate & Donor Relations Assistant

PARIS OFFICE | BARCELONA OFFICE | BRUSSELS OFFICE | R WORK REMOTELY | T TEMPORARY | C CONTRACTORS

INÉS HERNANDO
ERN & Healthcare Director 1/1

ANDREA CEMBRERO BONET
Patient Engagement Manager – Healthcare 1/1

NORA LAZARO
Patient Engagement Manager – Knowledge Management & Outreach – Healthcare 1/1

MATT JOHNSON R C
ERN & Healthcare Advisor, Mental Wellbeing Lead 2/5 (Köln)

CONCHA MAYO
Mental Wellbeing Engagement Manager 3/5 C

MICHAEL WILBUR

VIRGINIE HIVERT
Therapeutic Development Director 1/1

MARIA CAVALLER
Patient Engagement & Therapeutic Development Director 1/1

CLAUDIA FUCHS R
Drug Repurposing Project Senior Manager 1/1 (Bolzano)

JUDIT BAIJET
Patient Engagement & Training Manager 1/1

SHARON ASHTON R
Events and Open Academy Director, ECRD & BPA Leader 1/1 (London)

MARTINA BERGNA R
Events Senior Manager 1/1 (Milan)

MARTA CAMPABADAL
Open Academy Manager 1/1

TBR
Open Academy Training Manager 1/1

JESSICA KNOLLYS
Events Junior Manager 1/1

CELINE SCHWOB
Corporate Relations Director 1/1

JO MARSHALL (MAT LEAVE)
Corporate & Donor Relations Manager 1/1

ANNE-MARY BODIN
Resource Development Assistant 1/1

JESSIE DUBIEF
Social Research Director, Rare Barometer Programme Lead 1/1

FATOUMATA FAYE
Survey Project Manager 1/1

RITA FRANCISCO
Survey Project Junior Manager 1/1

EURORDIS' REPRESENTATION IN EXTERNAL NETWORKS, ORGANISATIONS AND INSTITUTIONS IN 2024

- **European Commission**
- **EU Health Policy Platform**
- Stakeholder Network for Rare Diseases
- Ukraine
- **Joint Research Center EU Platform Rare Diseases Registration (JRC)**
- **ACT-EU Accelerating Clinical Trials in the EU - stakeholder platform**
- **eHealth Stakeholder group**
- **EU Disability Platform**
- **Medecine Evaluation Committee (MEDEV) and Mechanism of Coordinated Access to orphan medicinal products (MoCA)**
- **Advisory Group on Raw Data**
- **European Medicines Agency (EMA)**
- 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
- **PRAC - Pharmacovigilance Risk Assessment Committee**
- **PCWP - Patients' & Consumers' Working Party**
- **SAWP - Scientific Advice Working Party**
- **CAT - Committee for Advanced Therapies**
- **CHMP - Committee for Medicinal Products for Human Use**
- **CTIS - Clinical Trial Information System Stakeholder and expert Group**
- **Topic Group on Patient Experience Data**

European Parliament

EURORDIS Network of Parliamentary Advocates for Rare Diseases

EU Cooperation on Health Technology Assessment (HTA)

Health Technology Assessment Stakeholder Network

European Reference Networks (ERNs) via ePAGs

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 SUPPORT TO NETWORKS AND ORGANISATIONS IN 2024

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

- 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)
- ARRIGE - The Association for Responsible Research and Innovation in Genome Editing
- TRANSFORM
- GO FAIR RD network
- EPF: European Patients' Forum (founding member) and EPF ehealth expert group
- EU4Health Civil Society Alliance (founding member)
- Rare Cancers Working Group of the European Society for Medical Oncology (ESMO)
- WECAN: Workgroup of European Cancer Patient Advocacy Networks
- ECO PAC: European Cancer Organisation Patient Advisory Committee
- EHA PAC: European Hematology Association Patient Advocacy 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)

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 research in rare diseases
- Genomics England

Partnership with Learned Societies:

- European Hospital & Healthcare Federation (HOPE)
- European Society of Human Genetics (ESHG)
- International Society for Pharmaco-economics and Outcomes Research (ISPOR)
- European Connected Health Alliance – ECHAlliance
- European Union of Medical Specialists (UEMS)
- European Alliance for Personalised Medicine
- European Association of Health Law
- European Federation of Internal Medicine (EFIM)
- Society for the Study of Inborn Errors of Metabolism (SSIEM)

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

- RDI: Rare Diseases International (founding member)
- WHO–Europe – Novel Medicine Platform (Steering Committee)
- International partnerships (MoUs): NORD (USA), CORD (Canada), JPA (Japan), RVA (Australia), CORD (China)
- IRDiRC: International Rare Disease Research Consortium (founding member)
- Global Commission to end the diagnostic odyssey for children (founding member)
- UDNI – Undiagnosed Diseases Network International
- NGO Committee for Rare Diseases (United Nations, New York) (founding member)
- IAPO: International Alliance of Patients' Organizations
- iCONS: The International Consortium on Newborn Sequencing
- PFMD - Patient Focused Medicines Development Initiative
- Ukraine Health Cluster (coordinated by the WHO)
- ORPHANET

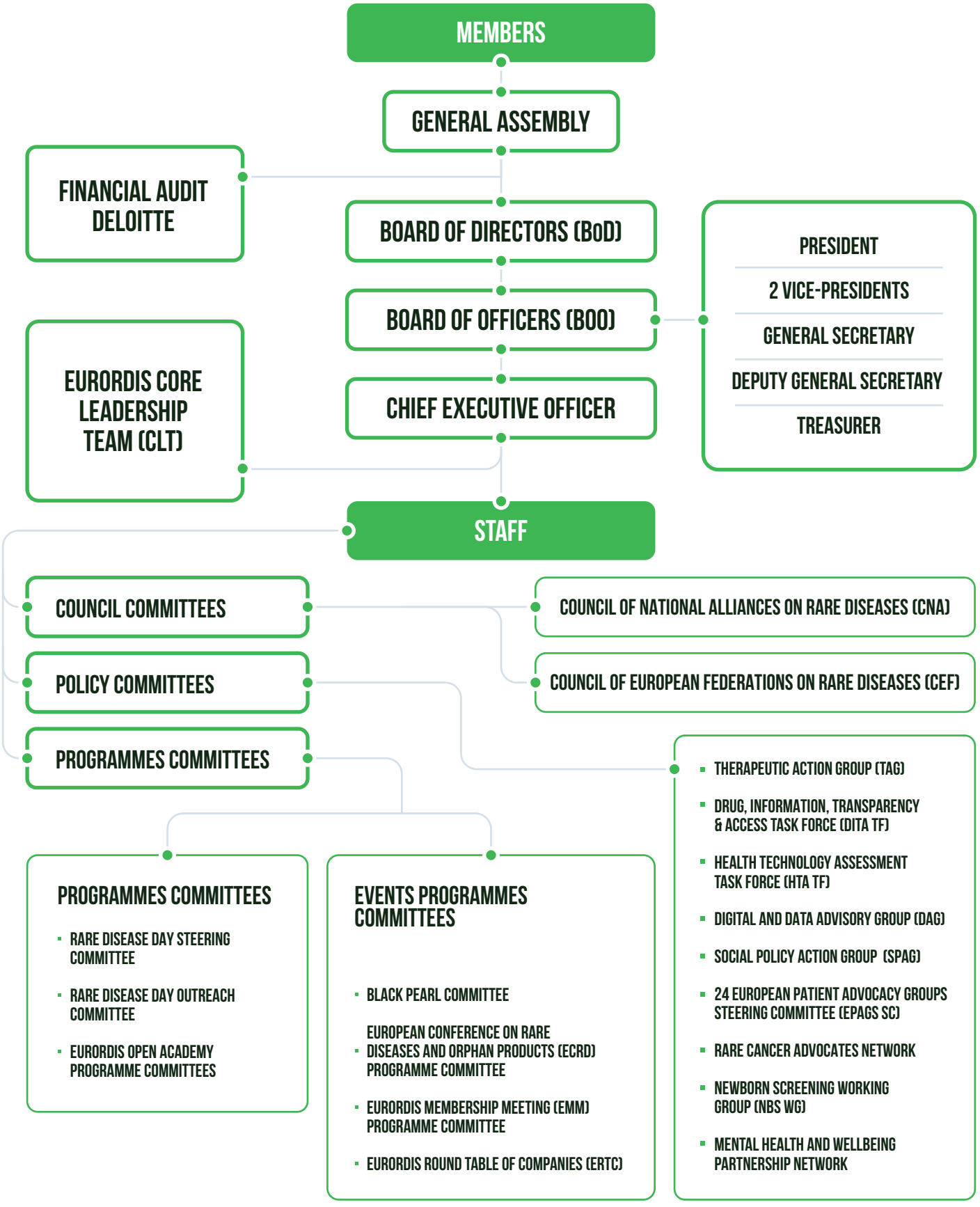
Member of European Projects:

- HTx - Next Generation Health Technology Assessment
- c4c - conect4children
- SolveRD - Solving the Unsolved Rare Diseases (end March 2024)
- EJP RD - European Joint Programme on Rare Diseases (end August 2024)
- 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
- 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

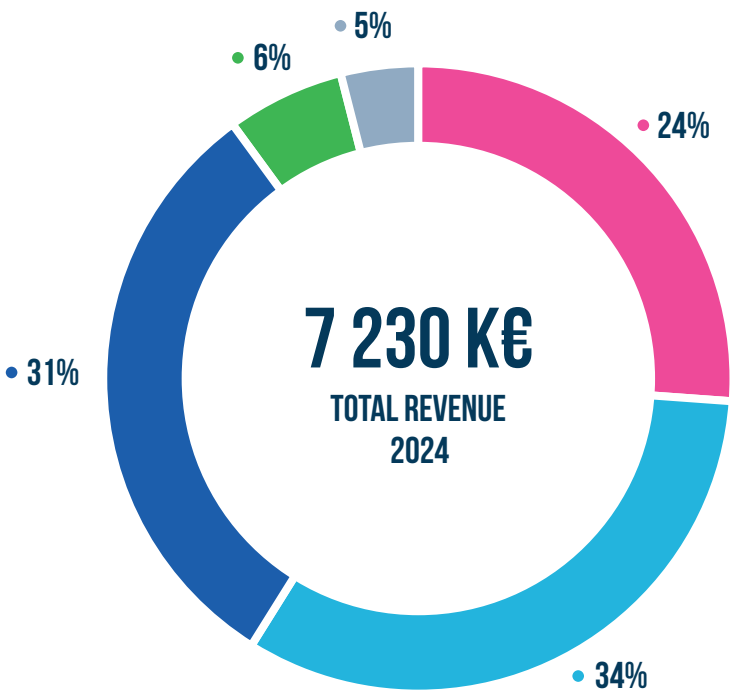
Steering Committees and Advisory Boards:

- BBMRI Stakeholders Forum
- HealthData@EU Pilot project - External Advisory Board
- Together4 Rare Diseases
- RWE4Decisions
- EuroCAB programme
- ERN-Hub for Ukraine

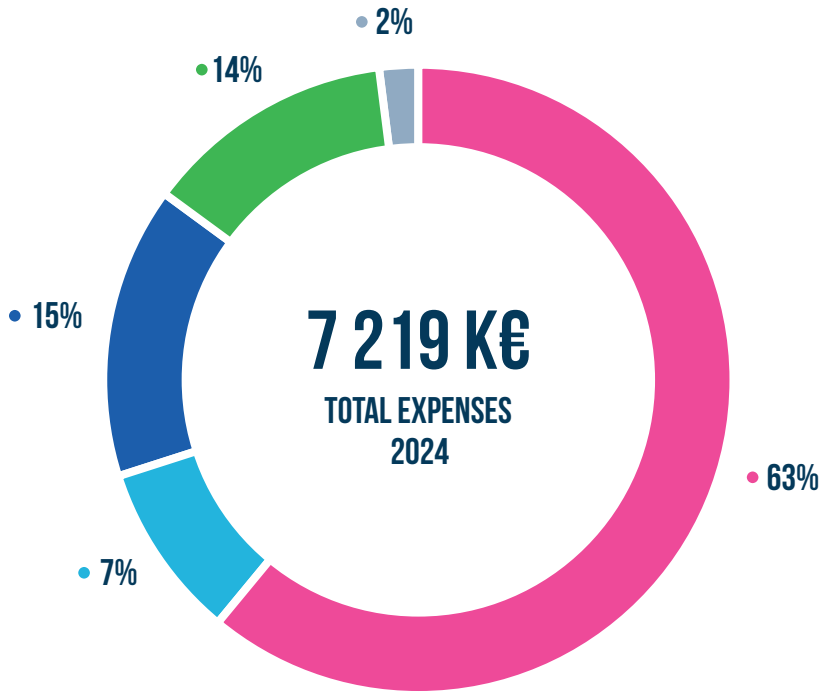
EURORDIS GOVERNANCE CHART 2024



REVENUES & EXPENSES 2024



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



LOGISTICS | VOLUNTEERS | STAFF COSTS | OTHERS | SERVICES

LEGEND

№	PAGE	DESCRIPTION
1	18	The RD Moonshot is a coalition of partners from industry, research and patient organisations joining forces to accelerate scientific discovery and drug development in rare and paediatric diseases for which currently there is no therapeutic option.
2	19	Rare Disease Partnership, a major research programme meant to start by late 2024.
3	23	FACILITATE's main objective is the development of a new ethical, legal, and regulatory framework to enable the return of clinical trial data to study participants and the healthcare professionals involved in their care. The secondary objective is to build a prototype process enabling the reuse of that data for future research needs.
4		Together4RD is a multi-stakeholder initiative aimed at supporting collaboration between European Reference Networks (ERNs) and industry in areas that will address the unmet medical needs related to the 95% of rare diseases without a dedicated treatment.
5	25	The STAMP project connects academic researchers with medicine regulatory agencies for early scientific advice on repurposing projects. This is instrumental in generating a robust data package to translate research into access to medicines for patients.
6		REMEDi4ALL aims to establish Europe's leadership globally in the repurposing of medicines by creating a vibrant community of practice covering all relevant sectors and disciplines.
7	27	Cavaller-Bellaubi, M., Hughes-Wilson, W., Kubinová, Š. et al. Patients, payers and developers of Orphan Medicinal Products: lessons learned from 10 years' multi-stakeholder dialogue on improving access in Europe via MoCA. Orphanet J Rare Dis 18, 144 (2023). https://doi.org/10.1186/s13023-023-02774-7
8	28	HTx aims to create a framework for the Next Generation Health Technology Assessment to support patient-centred, societally oriented, real-time decision-making on access to and reimbursement for health technologies throughout Europe.
9	29	GetReal Institute builds on the success of two IMI projects: GetReal and The GetReal Initiative and brings together a wide variety of stakeholders to drive the sustainable development and adoption of tools, methods and best practices in the generation and use of RWE (Real-World Evidence) for better healthcare decision-making.
10		VACCELERATE is a clinical research network for the coordination and conduct of COVID-19 vaccine trials. The network is comprised of academic institutions from all over Europe: The consortium is led by the University Hospital Cologne, Germany, and currently includes 29 national partners in 18 EU-member states and five countries associated to the EU Horizon 2020 research programme.
11		REMEDi4ALL aims to build a sustainable European Innovation Platform to enhance the repurposing of medicines. The project is expected to make a major leap forward in patient-centric drug repurposing, or finding new therapeutic options for existing drugs, in areas where there are high unmet medical needs, including the rare disease field.
12	30	The aim of Screen4Care is to accelerate the diagnosis of rare diseases based on two central pillars: genetic newborn screening and digital technologies.

EURORDIS
RARE DISEASES EUROPE

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EURORDIS
RARE DISEASES EUROPE

Plateforme Maladies Rares

96 rue Didot
75014 Paris
France

EURORDIS
BARCELONA OFFICE

Recite Modernista Sant Pau Pabellón de Santa Apolonia Calle Sant Antoni Me Claret 167

08025 Barcelona
Spain

EURORDIS
BRUSSELS OFFICE

Fondation Universitaire

Rue d'Egmont 11
1000 Brussels
Belgium

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WORK PROGRAMME
EURORDIS.ORG

2024