HYBRID

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3 - 4 JUNE 2026 • PRAGUE & ONLINE

13th EUROPEAN CONFERENCE ON RARE DISEASES AND ORPHAN PRODUCTS

RARE DISEASES IN A CHANGING & COMPETITIVE EUROPE

SHAPING POLICIES TO ADDRESS THE UNMET NEEDS OF PEOPLE LIVING WITH RARE DISEASES

ORGANISED BY:

EURORDIS
RARE DISEASES EUROPE

CO-ORGANISED BY:

orphanet
Inserm

WITH THE SUPPORT OF:

AFMTÉLÉTHON
INNOVER POUR GUÉRIR

#ECRD2026

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European Commissioner - Health and Food Safety

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PROGRAMME AT A GLANCE

ALL TIMES ARE CENTRAL EUROPEAN SUMMER TIME (CEST)

EVENT DESCRIPTION

DAY 1: WEDNESDAY 3 JUNE 2026

ALL TIMES LISTED ARE CENTRAL EUROPEAN SUMMER TIME (CEST)

- **09:00 – 10:30:** Opening Plenary
- **10:30 – 11:00:** Coffee Break and Free Networking
- **11:00 – 12:30:** Three Parallel Sessions – choose between:

  
  A1: Shaping the Future of Rare Disease Therapies:
  Innovation, Trials and Regulation in Europe's New Biotech Era

  
  B1: Early Detection & Newborn Screening (NBS):
  Feasibility, Cost and Equity

  
  C1: Advancing Holistic Care for Rare Conditions:
  A Patient-Centred and Evidence-Based Approach

- **12:30 – 14:00:** Lunch and Free Networking
- **12:45 – 13:45:** UCB Symposium: Navigating the Many Paths of the Diagnostic Odyssey
- **14:00 – 15:30:** Poster Pitches
- **15:30 – 16:30:** Networking Sessions
- **16:30 – 17:00:** Coffee Break and Free Networking
- **17:00 – 18:30:** Three Parallel Sessions – choose between:

  
  A2: Medical Devices for People Living with a Rare Disease

  
  B2: The Journey from Rare Disease Diagnosis to Treatment

  
  C2: Filling the Gaps to Provide Evidence-Based Holistic Care

- **18:30 – 19:30:** Welcome Reception

DAY 2: THURSDAY 4 JUNE 2026

ALL TIMES LISTED ARE CENTRAL EUROPEAN SUMMER TIME (CEST)

- **09:00 – 10:30:** Three Parallel Sessions – choose between:
  - D1: Strategies to Improve Access to Specialised Healthcare for People Living with a Rare Disease
  - E1: A State of the Art of the HTA Regulation (HTAR)
  - F1: Rethinking Mental Health in Rare Conditions: From Undefined Challenges to Collaborative Solutions
- **10:30 – 11:00:** Coffee Break and Free Networking
- **11:00 – 12:30:** Poster Pitches
- **12:30 – 14:00:** Lunch, Free Networking, and Corporate Donor Symposium
- **14:00 – 15:30:** Two Parallel Sessions – choose between:
  - D2: Rethinking Access to Specialised Healthcare for PLWRD – What Should We Measure?
  - E2: Preparing Reimbursement Decisions - Practical Consequences and Perspectives on the Future
- **15:30 – 16:00:** Coffee Break and Free Networking
- **16:00 – 17:15:** Closing Plenary

Therapies & Medical Devices, Development & Access

Diagnosis, Research and Prevention

Evidence-Based Holistic Care

Specialised Healthcare

Preparing Reimbursement Decisions

Mental Health

IN PARTNERSHIP WITH

WITH THE SUPPORT OF:

AFMTÉLÉTHON
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achse
Allianz Chronischer Seltener Erkrankungen

Alliance maladies rares
UNIS POUR VAINCRE

efpia
European Federation of Pharmaceutical Industries and Associations

ERDERA
European Rare Diseases Research Alliance

ESHG
EUROPEAN SOCIETY OF HUMAN GENETICS

EUCOPE
European Confederation of Pharmaceutical Entrepreneurs AISBL

EuropaBio
The European Association for Bioindustries

feder
FEDERACIÓN ESPAÑOLA DE ENFERMEDADES RARAS

MedTech Europe
from diagnosis to cure

Cyprus Alliance For Rare Diseases
C.A.R.D

rare diseases czech republic

RARE DISEASES INTERNATIONAL

Rare Diseases Ireland

RETOS LIGOS
NACIONALINIS ALJANSAS

ÜNIAMO
Federazione Italiana Malattie Rare

ASSOCIATE PARTNERS

BBMRI Biobanking and Biomolecular Resources Research Infrastructure

cam RARE Cambridge Rare Disease Network

CIRS Centre for Innovation in Regulatory Science

Childhood Cancer International Europe

conect 4children Collaborative Network for European Clinical Trials for Children

eatris

ECHO European Children's Hospitals Organisation

ecrin

EBC European Brain Council

FIPRA

Fondation Maladies Rares

Fondazione Telethon

institut imagine Guérir les maladies génétiques

ISNS International Society for Neonatal Screening

IRDiRC International Rare Diseases Research Consortium

The John Walton Muscular Dystrophy Research Centre

Medics for Rare Disease Excellence in rare disease medical training

Mental Health Europe

Newcastle Rare Disease Newcastle University Centre for Rare Disease

raremínds Mental Health for the Rare Disease Community

Screen 4 Care

UDN Undiagnosed Diseases Network

Multidisciplinary Joint Committee Rare and Undiagnosed Diseases U.E.M.S.

ERN ASSOCIATE PARTNERS

European Reference Networks
Endo-ERN
European Reference Network on Rare Endocrine Conditions

ERKNet
The European Rare Kidney Disease Reference Network

ERN BOND
EUROPEAN REFERENCE NETWORK ON RARE BONE DISEASES

EpiCARE

European Reference Network
for rare or low prevalence complex diseases
Network Hematological Diseases (ERN EuroBloodNet)

European Reference Network | ERN eUROGEN
Rare Urogenital Diseases & Complex Conditions

European Reference Network
for rare or low prevalence complex diseases
Network Neuromuscular Diseases (ERN EURO-NMD)

European Reference Network
ERN-EYE

European Reference Network
for rare or low prevalence complex diseases
Network Genetic Tumour Risk Syndromes (ERN GENTURIS)

European Reference Network
for rare or low prevalence complex diseases
Network Heart Diseases (ERN GUARD-HEART)

European Reference Network
for rare or low prevalence complex diseases
Network Intellectual Disability and Congenital Malformations (ERN ITHACA)

European Reference Network | Respiratory Diseases (ERN-LUNG)

European Reference Networks | ERN RARE-LIVER

European Reference Network | ERN ReCONNET

rita
Primary Immunodeficiencies / Autoinflammatory Disorders
Autoimmune Diseases / Paediatric Rheumatic Diseases

European Reference Networks | SKIN

TransplantChild
European Reference Network
for rare or low prevalence complex diseases
Network Transplantation in Children (ERN TRANSPLANT-CHILD)

European Reference Network
for rare or low prevalence complex diseases
Network Inherited and Congenital Anomalies (ERNICA)

European Reference Networks
for rare or low prevalence complex diseases
Network Neurological Diseases (ERN-RND)

European Reference Network | MetabERN
European Reference Network for Hereditary Metabolic Disorders

European Reference Network
VASCERN

OUTREACH COMMITTEE MEMBERS

Dorica Dan,
RONARD

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ASrid

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APARDO

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NORD

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Rare Diseases Portugal

Chris Higgins,
Rare Disorders New Zealand

Ulrike Holzer,
Pro Rare - Austria

Nick Meade,
Genetic Alliance UK

Ramaiah Muthyala,
Indian Organisation for
Rare Diseases

Liselotte Wesley,
Anderson Sjældne Diagnoser
(Rare Diseases Denmark)

Durhane Wong-Rieger,
Canadian Organisation for
Rare Diseases (CORD)

PARALLEL NETWORKING SESSIONS

NETWORKING SESSION 1:

DISCUSSION TABLES

You will have the chance to join focused round-table discussions organised by conference track, exploring specific sub-topics selected through a community call for proposals. Each table will be guided by a pre-assigned moderator, bringing together a diverse mix of participants from a range of different stakeholder groups, to ensure engaging, balanced, and interactive conversations.

NETWORKING SESSION 2:

CONNECTIONS BINGO

You will receive a bingo-style card, featuring both professional and general prompts, and move around the room to find peers who fit each prompt. Quick, energetic interactions will keep the session lively, helping you make multiple connections in a fun, accessible way, with small incentives for completing a row or the full card.

NETWORKING SESSION 3:

POSTERS SPEED DATING

You will receive a card featuring a series of numbers, with each number linking you to a poster on display in the on-site exhibition. Poster authors will be stationed next to their posters, ready to discuss their research with you. When the signal sounds, you will rotate to the next poster, allowing you to explore a wide range of research topics and engage directly with authors through a series of focused, interactive conversations.

THERAPIES AND MEDICAL DEVICES, DEVELOPMENT AND ACCESS TRACK

SESSION 1. WEDNESDAY 3 JUNE 2026, 11:00 – 12:30

SHAPING THE FUTURE OF RARE DISEASE THERAPIES: INNOVATION, TRIALS AND REGULATION IN EUROPE'S NEW BIOTECH ERA

SESSION DESCRIPTION

Rare diseases affect more than 300 million people worldwide, with significant economic and health impacts. At the same time, rare diseases are at the forefront of biotechnological innovation and investment in rare disease R&D is outpacing the broader biotech market, with a 28% year-on-year increase in funding and a growing pipeline of innovative therapies.

This session will explore how rare disease therapy development is being reshaped by technological innovation, global shifts, and forthcoming regulatory reforms. These changes are influencing how therapies are developed, tested, and delivered for underserved patient communities, opening new possibilities for people living with rare diseases (PLWRD).

The session will bring together perspectives from across the rare disease ecosystem to examine what these developments mean in practice, drawing on concrete examples of scientific advances, new regulatory approaches, and cross-border collaboration.

Participants in this session will consider how the implementation of the General Pharmaceutical Legislation revision and the proposed European Biotech Act could shape the next phase of rare disease innovation, with a focus on equitable access, regulatory agility, and a stronger European biotech ecosystem. The session will also spotlight initiatives uniting clinicians, regulators, industry, and patient organisations to accelerate the development of new therapies, including by co-creating innovative trial designs.

LEARNING OBJECTIVES

- Understand how the landscape of rare disease therapies, including emerging innovations and investment trends is being shaped by political, regulatory and technological shifts
- Explore challenges in clinical trials and novel approaches to trial design, collaboration, and stakeholder engagement
- Assess the impact of EU regulatory reforms and identify opportunities for patient-centred innovation and cross-sector collaboration

MODERATOR:

Tim Leest, Chair of the European Committee for Orphan Medicinal Products

SPEAKERS:

- **Antonio Parenti,** DG Santé, European Commission
- **TBC shortly**
- **TBC shortly**
- **TBC shortly**

SESSION 2. WEDNESDAY 3 JUNE 2026, 17:00 – 18:30

MEDICAL DEVICES FOR PEOPLE LIVING WITH A RARE DISEASE

SESSION DESCRIPTION

This session will highlight a unique and urgent opportunity to shape the future of medical device availability and access for people living with rare diseases, including children, in Europe. By the time of ECRD 2026, three major EU-funded projects—DeCODe, ORPHADEV4KIDS, and i4KIDS-4RARE—will be nearing completion. Each initiative addresses critical gaps in paediatric and orphan medical device development and has established collaborative platforms uniting clinicians, researchers, industry experts, and regulators to support the creation of safe, effective, and urgently needed devices.

Despite the life-changing potential of these devices and the launch of the Medical Devices Regulation, significant barriers remain. High costs, complex conformity assessments, and regulatory challenges threaten the development and continued availability of essential paediatric and orphan devices, leaving patients without adequate treatment options.

Recent European Commission guidance, a pilot programme and the proposed Regulation to simplify rules on medical and in vitro diagnostic devices, aim to prioritise orphan and paediatric devices for regulatory support and accelerate access. This session will explore how these advances can be translated into real-world impact, advocating for simplified regulatory pathways, continued support for innovation platforms, dedicated EU expert panels, and transparent post-market monitoring. Participants will have the opportunity to influence policy, support innovation, and help ensure that no individual living with a rare disease is left without the medical devices they need.

LEARNING OBJECTIVES

- Understand the landscape, gaps, and barriers in paediatric and orphan medical device development in Europe
- Explore EU initiatives, guidance, and pilot programmes that support innovation and accelerate access
- Identify strategies to improve policy, collaboration, and real-world access to safe and effective devices for children and rare disease patients

MODERATOR:

Dr Tomek Grybek, Foundation of Borys the Hero and EURORDIS Board Member

SPEAKERS:

- **Dr Donal O'Connor,** HPRA, and EC task forces on orphan devices and breakthrough technology
- **Dr Kolaleh Eskandian,** Compremium AG
- **Aleksandra Krygiel-Nael**, Johnson&Johnson
- **Dr Anneliene Jonker,** Duchenne Parent Project, and leader of DeCODe project
- **Dr Andrea Rappagliosi,** HTA Committee, Med-Tech Europe

DIAGNOSIS, RESEARCH AND PREVENTION TRACK

SESSION 1. WEDNESDAY 3 JUNE 2026, 11:00 – 12:30

EARLY DETECTION & NEWBORN SCREENING (NBS): FEASIBILITY, COST AND EQUITY

SESSION DESCRIPTION

Early detection is critical to improving outcomes for people living with rare diseases, yet newborn screening (NBS) programmes remain highly variable across Europe. While advances in genomics have expanded the potential of screening, many treatable rare conditions are still not systematically detected at birth.

This session will explore how screening and preventive approaches can function as front-line tools for earlier diagnosis and secondary prevention, enabling intervention before disease progression and reducing long-term health impacts. Discussions will consider how screening programmes can be designed to operate coherently across different stages of life, healthcare settings and health systems.

The discussions will focus on the feasibility, cost-effectiveness and equity of expanding screening programmes, drawing on national and international case studies. Speakers will explore emerging screening technologies, the role of data sharing and registries, and how evidence can inform decisions on programme expansion. Particular attention will be given to policy, ethical and legal considerations, including informed consent, data protection and equitable access across regions and populations.

By addressing financial, technological and governance challenges, this session will highlight how strengthened and harmonised screening strategies can reduce diagnostic delays, improve health outcomes, and ensure fair and sustainable access to early detection of rare diseases across Europe.

LEARNING OBJECTIVES

- Understand the role of screening and preventive approaches in the early detection and secondary prevention of rare diseases
- Assess feasibility, cost-effectiveness, ethical and policy considerations for expanding and integrating screening strategies within European health systems
- Identify best practices and lessons from national and international initiatives to support harmonised, equitable and sustainable approaches to early detection and prevention

MODERATOR:

Prof. Milan Macek, Head of Department of Biology and Medical Genetics, Charles University

SPEAKERS:

- **Martina Cornel, M.D, PhD,** European Society of Human Genetics
- **Sofia Douzgou Houge, M.D, PhD, FRCP,** Department of Medical Genetics, Haukeland University Hospital, Bergen, Norway
- **Prof. Dr Janbernd Kirschner,** Screen4Care
- **Dani Bancroft,** Genetic Alliance UK
- **Simon Lande,** Health Lumen
- **Dr Laurent Pasquier,** University Hospital Centre Rennes

SESSION 2. WEDNESDAY 3 JUNE 2026, 17:00 – 18:30

THE JOURNEY FROM RARE DISEASE DIAGNOSIS TO TREATMENT

SESSION DESCRIPTION

For many people living with rare diseases, the path from diagnosis to treatment is long and uncertain. Patients often face delays of five to seven years, multiple misdiagnoses, and limited access to genomic testing, delaying early intervention. Fragmented data systems, low awareness among health professionals, and uncertain genetic findings further complicate the transition to care.

This session will explore the real-world journey from diagnosis to treatment, highlighting systemic challenges and emerging solutions. Through case studies, speakers will examine how genomics, gene therapy, and digital innovation are transforming diagnostic and treatment pathways, while revealing practical, regulatory, and equity-related barriers. Presentations will cover gene therapy development and delivery for metachromatic leukodystrophy (MLD) and national experiences translating diagnosis into treatment. The session will also include short flash presentations showcasing AI and digital tools in diagnostics. The session will also highlight the essential role of post-diagnostic genetic counselling in enabling informed decisions, care planning, and access to emerging therapies.

By linking scientific progress, digital innovation, patient experience, and health system realities, the session will show how integrated, timely, and equitable pathways can bring innovative therapies to patients who need them most.

LEARNING OBJECTIVES

- Identify key barriers delaying rare disease diagnosis-to-treatment pathways, including diagnostic odysseys and limited genomic access.
- Explore how genomics and gene therapy are transforming care through real-world case studies and country examples.
- Understand the clinical, regulatory, and equity challenges in bringing innovative therapies to patients across Europe.

MODERATOR:

Dr Gulcin Gumus, EURORDIS - Rare Diseases Europe

SPEAKERS:

- **Urh Groselj, M.D, PhD,** University Medical Centre Ljubljana
- **Prof. Kelly Ormond,** ETH-Zürich and Stanford
- **Dr Andreas Øberg,** Oslo University Hospital

FLASH PRESENTERS:

- **Prof. Jean Bergounioux,** Raymond Poincaré Hospital, APHP
- **Prof. Roman Hossein Khonsari,** AIDY
- **Dr Ana Rath and Robin Safarti,** Rare Disease Knowledge (RDK)

EVIDENCE-BASED HOLISTIC CARE TRACK

SESSION 1. WEDNESDAY 3 JUNE 2026, 11:00 – 12:30

ADVANCING HOLISTIC CARE FOR RARE CONDITIONS: A PATIENT-CENTRED AND EVIDENCE-BASED APPROACH

SESSION DESCRIPTION

Most people living with a rare disease do not expect a cure by 2030 but believe their quality of life can be significantly improved through better access to holistic care. Evidence from the Rare Barometer and Rare 2030 survey on the future of rare diseases (2021) shows that care coordination and psychosocial support remain among the highest unmet needs, highlighting the demand for care models that integrate clinical, psychological and social dimensions.

This session will explore what holistic care means in practice for people living with rare diseases and how it can be better understood, evidenced and implemented. Speakers will present existing evidence on holistic care delivery, identify gaps in patient involvement and policy, and examine how patient-generated data—such as surveys, health-economics studies and patient-reported experience measures (PREMs)—can strengthen decision-making and service design.

Through case studies and interactive discussions, the session will consider how evidence can be used to improve coordination, inform policy, and support more patient-centred and integrated approaches at local, national and European levels. By bringing together patients, clinicians, researchers and policymakers, the session aims to advance shared understanding and collaboration towards scalable, evidence-based holistic care for rare conditions.

LEARNING OBJECTIVES

- Capture patient expectations and needs regarding holistic care for people living with rare diseases.
- Review evidence on how holistic care is measured and delivered.
- Identify key gaps in patient involvement, policy, and care coordination.
- Explore data and tools (e.g. PREMs) that support evidence-based care.
- Encourage collaboration among patients, clinicians, researchers, and policymakers to improve holistic care.

MODERATOR:

Claudia Crocione, HHT Europe

SPEAKERS:

- **Jessie Dubief,** EURORDIS – Rare Diseases Europe
- **Iolo Eilian,** HSE Ireland
- **Dr Roser Francisco,** Hospital Sant Joan de Deù
- **Dr Rohita Sharma,** Alexion
- **TBC shortly**

SESSION 2. WEDNESDAY 3 JUNE 2026, 17:00 – 18:30

FILLING THE GAPS TO PROVIDE EVIDENCE-BASED HOLISTIC CARE

SESSION DESCRIPTION

There is a strong demand for holistic care for people living with rare diseases—care that integrates clinical, psychological, and social dimensions. Many patients see better care coordination and support for the psychosocial impacts of their condition as top priorities, yet pathways to achieve this remain uncertain. More evidence is needed to determine what works, what can be scaled, and how to embed patient perspectives into care, research, and policy.

This session will explore ways to address current gaps in delivering evidence-based holistic care. Discussions will focus on how rare disease registries and national databases can support non-clinical evidence generation, including social, economic, and psychosocial data, and how research in the social sciences and humanities (SSH) can inform better understanding and delivery of holistic care. Participants will also examine opportunities for transnational collaboration, funding, and training to strengthen patient-informed approaches.

By linking registry data, SSH research, and patient perspectives, the session will highlight practical strategies to improve care coordination, support psychosocial needs, and foster patient-centered, evidence-based practices. Attendees will gain insights into bridging key evidence gaps through interdisciplinary and cross-border approaches, while exploring ways to enhance collaboration among patients, researchers, clinicians, and policymakers across Europe.

LEARNING OBJECTIVES

- Identify current evidence gaps hindering the implementation of holistic care for people living with rare diseases.
- Explore how existing registries and national databases can support non-clinical evidence generation, including social, economic, and psychosocial data.
- Showcase the contribution of social sciences and humanities (SSH) research to better understanding and delivery of holistic care.
- Discuss opportunities for transnational collaboration, funding, and training to strengthen evidence-based, patient-informed care.

MODERATOR:

Jessie Dubief, EURORDIS – Rare Diseases Europe

SPEAKERS:

- **Dr Pauline Nauroy,** Fondation Maladies Rares
- **Dr Solange Roumengous,** APHP – BNDMR France
- **Eva-Maria Strömsholm,** Finnish Association of Gynaecological Cancer Patients
- **Dr Natalie Uhlenbusch,** University Hospital Hamburg - Q.RARE.LI Project
- **TBC shortly**

SPECIALISED HEALTHCARE TRACK

SESSION 1. THURSDAY 4 JUNE 2026, 09:00 – 10:30

STRATEGIES TO IMPROVE ACCESS TO SPECIALISED HEALTHCARE FOR PEOPLE LIVING WITH A RARE DISEASE

SESSION DESCRIPTION

Ensuring equitable access to timely, high-quality specialised healthcare remains a critical priority for people living with rare diseases (PLWRD). This requires systematically addressing financial, linguistic, mobility, and administrative barriers that impede equitable access, as well as establishing clear referral pathways from frontline services to Expert Centres at both national and European levels. This session will explore how health systems can optimise national and cross-border access to specialised healthcare through innovative organisational arrangements.

Speakers will discuss national and EU-level strategies to improve and accelerate access to speciliased healthcare services, including the role of National Reference Networks, ERNs National Coordination Hubs, and generic care pathways for rare complex conditions. The session will highlight how patients experience access to specialised healthcare, drawing on evidence from surveys and patient-reported priorities.

Through a patient perspective and panel discussion, the session will showcase practical approaches to enabling navigation of complex health systems. Participants will explore actionable strategies to ensure PLWRD can access the right care, from frontline services to leading expert centres, in a timely and equitable manner.

LEARNING OBJECTIVES

- Examine how health systems may improve and accelerate access to specialised healthcare for people living with a rare or complex condition.
- Understand real-world experiences to improve access and what works in different health system contexts.
- Discover strategies to connect the national and EU levels, and what role can Rare Disease National Alliances play to improve access.

CO-CHAIR:

Vicky Hedley, Newcastle University

SPEAKERS:

- **Cecilia Gunnarsson M.D.,** Linkoping University
- **Anna Arellanesová,** Rare Diseases Czech Republic
- **Prof. Luca Lovrečić,** University Medical Centre Ljubljana
- **Dr Holm Graessner,** University Hospital Tübingen

SESSION 2. THURSDAY 4 JUNE 2026, 14:00 – 15:30

RETHINKING ACCESS TO SPECIALISED HEALTHCARE FOR PLWRD – WHAT SHOULD WE MEASURE?

SESSION DESCRIPTION

Ensuring timely access to high-quality specialised healthcare is a top priority for people living with rare and complex conditions. While many services are delivered in tertiary centres by multidisciplinary teams, patients often face long waits, diagnostic delays, and need to travel nationally or across borders to access these services, but travel and logistics expenses are not covered by health systems.
Current health system assessments may not fully capture these challenges or the impact of interventions on health outcomes.

This session will focus on performance assessment and key metrics to better inform policy and decision-making. Speakers will explore which indicators different countries are using to monitor access to specialised healthcare, assess unmet needs, and evaluate the impact of organisational arrangements such as care pathways, Centres of Expertise, and cross-border healthcare services. The session will highlight national evaluation and monitoring practices and how to leverage existing health information systems to generate actionable evidence for rare disease policy.

Through a keynote speech followed by a panel discussion, participants will explore how health systems can enhance their current measurement frameworks by linking structural indicators with performance, outcomes, and patient experiences. The session will examine which key metrics should be integrated into decision-maker dashboards to provide actionable intelligence on access to specialised healthcare for rare diseases.

LEARNING OBJECTIVES

- Understand how health systems currently monitor access to specialised healthcare for people living with a rare condition, including gaps and the use of outcome measures
- Explore how health systems performance assessment can inform policy making and improve equitable access to specialised healthcare services
- Identify indicators and metrics to create a dashboard for decision makers to better monitor and evaluate access to specialised healthcare

CO-CHAIRS:

- **Dr Enrique Terol,** Permanent Representation of Spain to the EU
- **Ines Hernando,** EURORDIS – Rare Diseases Europe

KEYNOTE SPEAKER:

Dr Marina Karanikolos, European Observatory on Health Systems and Policies

PANEL SPEAKERS:

- **Dr Anne-Sophie Lapointe,** DGOS, French Ministry of Health
- **TBC shortly**
- **Charline Maertens,** KCE Belgian Health Care Knowledge Centre

PREPARING REIMBURSEMENT DECISIONS TRACK

SESSION 1. THURSDAY 4 JUNE 2026, 09:00 – 10:30

A STATE OF THE ART OF THE HTA REGULATION (HTAR)

SESSION DESCRIPTION

The Regulation (EU) 2021/2282 on Health Technology Assessment (HTAR) is transforming how medicines, including orphan and advanced therapy medicinal products (ATMPs), are assessed across Europe. This session will provide a state-of-the-art overview of the HTAR, focusing on its key components, experiences since it entered into force, and lessons learned in its first 18 months of implementation.

Speakers will review joint clinical assessments (JCAs), joint scientific consultations (JSCs), and European reports, exploring what has worked well, where challenges remain, and how processes have adapted. Perspectives will include those of industry and clinical experts, highlighting successes in engagement, preparation, and expert participation, as well as challenges specific to orphan products, such as small patient populations, scattered expertise, and managing potential conflicts of interest.

Depending on how many JCA reports will be made available to Member States by May 2026, the session will also examine the use and impact of European joint reports at national level, and whether their conclusions are clear, actionable, and integrated into local reimbursement and policy decisions. By reflecting on these experiences, participants will gain insights into how the HTAR can better accommodate the specific needs of rare disease treatments, and how stakeholders, including people living with rare diseases, can be effectively involved as the regulation's scope expands to orphan products in 2028.

LEARNING OBJECTIVES:

- Analyse lessons learned from early HTAR joint work, including successes and challenges from oncology products and ATMPs
- Define meaningful patient involvement in joint clinical assessments and scientific consultations
- Explore practical approaches to evidence generation and assessment in preparation for including orphan diseases within the HTAR scope

MODERATOR:

François Houÿez, EURORDIS – Rare Diseases Europe

SPEAKERS:

- **Camelia Isaic,** HAE Junior
- **Maciej Gajewski,** Ipsen
- **Dr Stephanie Said,** HTA Coordination Group Subgroup on JSC, Federal Joint Committee, Gemeinsamer Bundesausschuss (G-BA)

SESSION 2. THURSDAY 4 JUNE 2026, 14:00 – 15:30

PREPARING REIMBURSEMENT DECISIONS - PRACTICAL CONSEQUENCES AND PERSPECTIVES ON THE FUTURE

SESSION DESCRIPTION

This session will provide a forum for key stakeholders to discuss the practical consequences of the Health Technology Assessment Regulation (HTAR) and to share perspectives on its future implementation. Building on experiences with oncology products and advanced therapy medicinal products (ATMPs), speakers will explore how European joint assessments are being used at the national level, and whether they are facilitating discussions and decision-making among stakeholders.

The session will consider criteria and indicators for monitoring the success of the HTAR, including uptake of joint reports by Member States and the evolving work of subgroups. Participants will discuss lessons learned from current assessments, particularly for orphan and ultra-rare diseases, including the potential need to adapt processes such as the submission of PICO questions or the use of real-world data and evidence.

Stakeholder perspectives—including industry, clinical experts, and patient representatives—will highlight challenges and opportunities, including meaningful patient engagement, alignment of joint reports with national decision-making, and strategies to prepare for the inclusion of orphan diseases and medical devices within the HTAR scope in 2028.

By reflecting on these experiences and identifying priorities for improvement, the session will support stakeholders in ensuring that the HTAR effectively addresses the needs of rare disease populations while guiding future revisions of the regulation.

LEARNING OBJECTIVES

- Understand the impact of the HTAR on rare and complex conditions, including stakeholder perspectives and national decision-making
- Examine patient involvement in joint assessments and approaches tailored to orphan and ultra-rare diseases
- Discuss evaluation, improvement, and future adaptation of HTAR processes to support timely access to rare disease treatments

MODERATOR:

Julien Delaye, Brussels Center for Collaboration in Health (BCCH)

SPEAKERS:

- **Dr Roisin Adams,** National Centre for Pharmacoeconomics, Ireland
- **Gaetan Duport,** European Haemophilia Consortium
- **François Houÿez,** EURORDIS – Rare Diseases Europe
- **Matteo Scarabelli,** EFPIA
- **Dr Jose Valverde,** DG Santé, European Commission

MENTAL HEALTH TRACK

THURSDAY 4 JUNE 2026, 09:00 – 10:30

RETHINKING MENTAL HEALTH IN RARE CONDITIONS: FROM UNDEFINED CHALLENGES TO COLLABORATIVE SOLUTIONS

SESSION DESCRIPTION

Mental health challenges for people living with rare diseases (PLWRD) are often misunderstood, under-researched, and excluded from integrated care models. For many rare conditions, mental health is not only a co-morbidity but a primary feature, shaped by biological, psychological, and social factors. Families face a "black hole" of support when young people with intellectual disabilities age out of paediatric systems, while adults often lack access to structured neuropsychological assessments.

At the clinical and research levels, gaps persist in definitions, biomarkers, and trial designs that account for mental health complications. Socially, caregivers experience profound stress, isolation, and economic strain, frequently with little guidance or systemic support. Fragmentation across sectors leaves patients and families vulnerable, without clear pathways to holistic and psychologically informed care.

The session aims to rethink mental health in rare conditions by clarifying definitions, addressing systemic gaps, and forging cross-sector solutions that embed psychological and neuropsychological support as a core component of biopsychosocial care. It will provide a critical space to elevate mental health as a European policy priority, highlighting the urgent need for integrated strategies across clinical care, research, and social systems. Participants will explore practical approaches to close gaps, strengthen coordination, and ensure mental health is recognised and supported alongside physical health in rare disease care.

LEARNING OBJECTIVES

- Explore distinct challenges: Understand how mental health for PLWRD differs from the general population, including for transitional care issues and unique social burdens.
- Identify systemic gaps: Highlight fragmented transitional pathways.
- Advance integrated models: Showcase emerging practices that combine biomarkers, clinical trials, and family-centred care.
- Foster collaboration: Encourage multidisciplinary and cross-country approaches, building momentum toward a shared European strategy.

CHAIR:

Kirsten Johnson, Fragile X International

SPEAKERS:

- **Isabella Brambilla,** Dravet-Italia, CREA & EpiCARE
- **Jessie Dubief,** EURORDIS – Rare Diseases Europe
- **Vinciane Quoidbach,** European Brain Council
- **Dr Stewart Rust,** Manchester University NHSFT
- **Dr Jane Waite,** Cerebra Network

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ORGANISED BY:

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