# GOOD HEALTH AND WELL-BEING

**GOAL LEADERS:**

**Michela Gabaldo**, Fondazione Telethon

**Holm Graeßner**, ERN RND and University of Tübingen

**Victoria Hedley**, Rare 2030 and University of Newcastle-upon-Tyne

**Elizabeth Vroom**, Global Duchenne Foundation and EURORDIS

**With the support of:**

**Matt Bolz-Johnson**, EURORDIS

**Inés Hernando**, EURORDIS

## Goal description

The 3rd Sustainable Development Goal to *ensure healthy lives and promote well-being for all people living with a rare disease at all* ages is closely linked to the concept of Universal Health Coverage (UHC). Achieving UHC is about having timely access to diagnosis as well as access to effective, responsive, safe and affordable care and treatments to ensure better health outcomes.

The rare disease community envisions a future where, with increased solidarity, countries will overcome healthcare fragmentation and inequalities in access, through interconnected and comprehensive health & social care systems that answer to the evolving needs faced by the 30 million people living with a rare disease in Europe.

While the Rare2030 Foresight Study provides the overall direction to achieve such a future in the next decade, the rare disease community still needs to define the specific organisational and institutional arrangements that will move us from the aspirational scenario depicted in these recommendations to transforming care delivery and achieving better health outcomes. This goal will on the hand, identify specific measures to strengthen health systems arrangements to address some of the wellknown access challenges faced by people living with a rare disease. On the other hand, it will address emerging challenges that call for a deeper transformation of our current health systems propose innovative solutions to tackle these.

The sessions under Track A will focus on well-known "access" challenges and explore immediate measures, low hanging fruits, to increase access to effective diagnostic tools, to highly specialised healthcare services and to allied treatments and interventions for all people living with a rare disease across Europe. Whereas Track B will be devoted to imagining new solutions for emerging challenges and to envision pan-European arrangements, under a future European Health Union, for the delivery of highly specialised healthcare for ultra-rare diseases, as well as the delivery of advanced therapy medicinal products (ATMPs).

Each track will feature a workshop showcasing good practices and evidence, followed by a policy formulation session workshop. The workshops and sessions will address jointly upstream (screening and diagnosis) and downstream (healthcare services and treatments) aspects to formulate policy options that consider as much as possible the interdependencies of upstream and downstream associated health services.

Other key components of UHC such as financial protection, well-being promotion and social inclusion will be addressed by the sessions under Goal 2 and Goal 3.

## Track A: Strengthening national health systems to improve access to effective diagnostic testing technologies, care and treatments.

**Session 1:** Tuesday 28 June 2022, 14:00 – 15:30 CET

### Good practice and evidence

This session will present evidence and good practice along the continuum of care to inspire short-term policy action at EU and/or national level. Participants will learn what are the main personal and external factors affecting patients' access to diagnosis, according to the findings of the latest Rare Barometer Voices survey on diagnosis. They will also discover how well-designed care pathways, that optimise access to rare disease Expert Centers, may improve patients' experience with care.

Several case studies will showcase how rare disease Expert Centres can arrange care continuity and coordination with social services and will present innovative approaches to improve access to allied therapies for people living with a rare disease.

**Chaired by**
**Elizabeth Vroom,** Chair World Duchenne Organization and EURORDIS Board member

**Speakers**
**Sandra Courbier,** Social Research Director - Rare Barometer Programme Lead, EURORDIS
**Sanne Bouwman,** Marketing and communication advisor, ParkinsonNet
**Julie Vallortigara,** Research Fellow, Department of Clinical and Movement Neurosciences, University College London
**Vinciane Quoidbach,** European Brain Council
**Johanna Blom,** Physiotherapist, Neurology Department of the University Hospital of Skåne in Malmö, Sweden
**Monika Benson,** Executive Director, Dystonia Europe
**Eduard Pellicer,** Social Worker - Chronic Complex Patients, Hospital Sant Joan de Déu, Barcelona

**Session 2:** Tuesday 28 June 2022, 16:00 – 17:30 CET

## Policy formulation session

This session will build on the experiences and insights shared in Session 1 to propose concrete policy recommendations to improve access to i) effective diagnostic technologies and services, ii) highly specialised healthcare and iii) allied health and therapy services.

Speakers will identify specific measures to remove the barriers to access diagnostic technologies for rare diseases and strengthen health systems capacities to treat the increasing number of people that are being diagnosed. The panel will also discuss policy and organisational measures to optimise the design of care pathways to accelerate access to expert care as well as ways to ensure continuous and timely access to allied health and therapy services.

**Chaired by**
**Holm Graessner**, Head Research Management Unit, COO Centre for Rare Diseases Tübingen & Coördinator, ERN-RND.
**Elizabeth Vroom,** Chair World Duchenne Organization and EURORDIS Board member

**Speakers**
**Laurence Faivre**, Professor at the University of Bourgogne, Hospital Practitioner
**Hortensia Gimeno**, Associate Director for Research and Clinical Effectiveness, National Health Service of United Kingdom
**Holm Graessner**, Head Research Management Unit, COO Centre for Rare Diseases Tübingen & Coördinator, ERN-RND.
**Eileen Treacy,** Clinical Lead, National Rare Diseases Office

## Track B: Inventing innovative health systems arrangements to address new and emerging challenges.

**Session 1:** Tuesday 28 June 2022, 14:00 – 15:30 CET

## Good practices and evidence

This session will showcase a range of good practices in the broad area of 'innovation across the care pathway', starting from accelerated applications of genomic technologies, expansion of newborn screening panels for actionable diseases, and integration of diagnostic approaches for undiagnosed cases, all of which should serve to facilitate an accurate and timely diagnosis and offer the opportunity for all PLWRD to be fast-tracked on an effective pathway.

Larger populations of accurately diagnosed individuals must be accompanied by equally innovative approaches to 'open the door' to curative and transformative therapies; therefore, this session will also consider innovation in health systems to deliver and access highly

specialised healthcare services for very rare diseases, intended to speed up the rate at which innovative therapies become accessible to the rare disease community.

**Chaired by**
**Victoria Hedley**, Policy Officer, RARE 2030 Foresight Study & University of NewcastleuponTyne.

**Speakers**
**Mark Briggs**, Assistant Director of Innovation, Cardiff and Vale University Health Board, United Kingdom
**Marie-Christine Ouillade**, Board of Directors, AFM-Téléthon & SMA Europe
**Tudor Groza**, Phenomics Team Lead, European Bioinformatics Institute
**Olaf Riess,** Head of the Institute of Medical Genetics and Applied Genomics

**Session 2:** Tuesday 28 June 2022, 16:00 – 17:30 CET

## Policy formulation session

'Healthy lives' and greater wellbeing for people living with rare diseases is only possible through a coordinated policy approach which unlocks the potential of innovation in multiple domains, including digital systems, genomic technologies, health systems infrastructure, manufacturing processes, workforce development, knowledge building, and sharing data. Besides spanning multiple disciplines, policies to support innovation in rare disease health systems must often also span many geographical borders.

Building on the good practices and insights shared in Session 1, a panel of experts will discuss policy opportunities to scale-up innovation to ultimately increase life expectancy and improve quality of life for the 30 million Europeans affected by a rare disease or rare cancer (as opposed to the few).

Recognising the need for concerted action for rare disease, the session will discuss some of the core building blocks for a new strategic policy framework for rare diseases, identifying where innovation can be better leveraged to allow EU citizens to benefit from innovative and curative therapies. The pros and cons -and feasibility- of different approaches will be discussed, by considering underdeveloped tools, programmes and approaches which already exist, whilst contemplating what radical future policies might look like.

The session will explore if the existing health legal and institutional framework would allow the flexibility to keep pace of innovation developments as well as fleshing out greater informal or formal collaboration across the European Union, centralising planning and organisations of services, taking a whole-system approach to accelerate adoption and access of innovation into frontline services, access to innovation to all.

**Chaired by**
**Matt Bolz-Johnson,** Healthcare Advisor, EURORDIS

**Speakers**
**Nick Meade**, Joint Interim Chief Executive & Policy Director, Genetic Alliance UK
**Eva Schoeters,** Director, RaDiOrg- Rare Disease Organisation Belgium
**Giancarlo La Marca**, Deputy Head of the Newborn Screening, Clinical Chemistry and Pharmacology Laboratory, Meyer Children's Hospital, Italy
**Wendy van Zelst-Stams**, Clinical Geneticist, Radboud University Medical Centre, Netherlands
**Victoria Hedley**, Rare Disease Policy Manager, Newcastle University

**Goal description**

# REDUCED INEQUALITIES

**GOAL LEADERS:**

**Matt Bolz-Johnson**, EURORDIS

**Ana Rath**, Orphanet

**With the support of:**

**Valentina Bottarelli**, EURORDIS

**Clara Hervas**, Edelman

'Systems' are not adapted to the complex needs of people living with a rare disease (PLWRD). Focusing on reducing inequalities for PLWRD involves the identification and the removal of systemic barriers through targeted policy interventions to ensure PLWRD are given the same opportunities. Focusing on reducing inequalities for PLWRD means achieving greater equity, by providing appropriate compensatory policy provisions to eliminate disadvantage and support affected individuals in reaching their maximum level of health and well-being. For this, a holistic approach that looks at the interrelated aspects of physical, mental, and social health and well-being, and that encompasses the entire life-course, is needed.

A major barrier to improving the inclusion and participation of PLWRD and their families in society is the scarcity of knowledge, limited expertise on the field and a lack of awareness of rare diseases and understanding of their impact. As a consequence, this population is psychologically, socially, culturally and economically vulnerable and faces discrimination and specific challenges in health and social care, education, employment and leisure. This, in turn, causes increased impoverishment and isolation. PLWRD can experience this at any point, or throughout, the course of life and additional factors such as gender, living in a rural area or being from a racial or ethnic minority can worsen the challenges. The COVID-19 pandemic has also had a great and disproportionate impact on PLWRD, with already ill-adapted systems being stretched even further. Our most vulnerable in society should be at the heart of any health emergency response, but instead their needs are too often traded-off and left unaddressed, resulting in life-long consequences.

Greater equity for PLWRD is prompted by the removal of these barriers. Equity is founded in cultural and societal values that are essential in order to ensure the rights of PLWRD are upheld. Equity is central to the rights to life, liberty, security of person and cuts across the whole spectrum of the pillars of our society, from education to work, offering the same opportunities for PLWRD to secure an adequate standard of living as well as to enjoy the highest attainable standard of physical and mental health. In addition, focusing on equity for PLWRD can unlock society-wide public good, benefitting not only the millions of PLWRD and their families but also bringing a productive population back into economies as well as crucial improvements in health and social care and social innovation beyond just the rare diseases field.

The complex and holistic needs of PLWRD are gaining in political recognition and support, with the unanimous approval by the United Nations' General Assembly in a new resolution aim at tackling the challenges of people living with a rare disease and their families. The new political endorsement by the UN offers the rare disease community the opportunity to target systemic change and policy development and closing the equity gap between individuals and communities once and for all!
Goal 2 of ECRD 2022 aims to explore the many opportunities to improve the recognition, understanding and knowledge of rare diseases and their impact on the people who live with them,

spearheaded by policy interventions that can address structural barriers in accessing health and social care, and empower self-determination to live life independently through access to education and work.

Track C & D Session 1 sets the framework to navigate the following two topic-based sessions. It considers how rare diseases are invisible in ill-adapted health care and social systems and addresses the preconditions for inclusions and equal opportunities for PLWRD.

Track C Session 2 explores how greater integration of health and social care unlocks better access to health and social care for people living with a rare disease.
Track D Session 2 shows the everyday challenges for a PLWRD to access education and employment while sharing good practices of inclusivity at school and work which trigger individuals' greater independence and contribution to society.

Across all Sessions, case studies will be presented whereas panel discussions will formulate policy solutions.

**Track C/D Session 1:** Wednesday 29 June 2022, 14:00 – 16:00 CET

## Invisibility as a roadblock towards reducing inequalities for people living with a rare disease.

People living with rare diseases (PLWRD) are invisible in ill-adapted systems due to the lack of knowledge, recognition and understanding on rare diseases and their impacts on all aspects of life. Inequalities in health-social care, education and work are majorly due to the lack of adaptation to the needs of this population. The greater the complexity, the greater the need for the service-system-society to adapt, and this depends on a combination of awareness, knowledge, willingness as well as tools and practices that empower positive action.

Inequalities experienced by PLWRD can be made visible through disability assessment, at an individual level, and by measuring the burden of rare diseases, at a population level. However, methodologies and practices do not always capture and recognise the complex needs of PLWRD, consequently limiting access to benefits, social protection, and reasonable accommodation.

This session sets the framework to navigate the following two topic-based sessions within Goal 2 of the programme. With the premise that visibility is a pre-condition for inclusion of PLWRD in society, it outlines the evidence and data needed to measure the burden of rare diseases to make them more visible and to inform policy decisions. The session also outlines the infrastructural, technological and policy shifts and tools that support systems to adapt to the needs of PLWRD, and that favour inclusion and equal opportunities in society (policy frameworks like for instance, the European Pillar of Social Rights or the Disability Strategy).

**Chaired by:**
**Dr Ana Rath**, Director, Orphanet.

**Speakers:**
**Dr Juanita Haagsma,** Assistant Professor, Erasmus Medical Centre Rotterdam
**Dr Androulla Eleftheriou,** Executive Director, Thalassaemia International Federation
**Dr Ana Rath,** Director, Orphanet
**Flaminia Macchia,** Executive Director, Rare Diseases International
**Alba Ancochea,** Chief Executive Officer, FEDER (Federacion Espanola de Enfermedades Raras)
**Inmaculada Placencia Porrero,** Senior Expert, European Commission - DG Employment, Social Affairs & Inclusion
**Dr Anne-Sophie Lapointe,** Head of Rare Diseases Project, French Ministry of Health and Solidarity

**Track C Session 2:** Wednesday 29 June 2022, 16:30 – 18:00 CET

## Inequalities in accessing health and social care for people living with a rare disease.

People living with a rare disease need medical care and follow-up support from different categories of health professionals, often from several different medical specialties, as well as from social workers and other social and local service providers. However, people living with a rare disease face fragmented care pathways and mainstream services that are not flexible enough to take into consideration unprecedented needs.

Health and social care services are not constructed in a way to meet the needs of complex diseases and development disorders. Services have structural barriers to access services leading to vulnerable individuals falling through the gaps in health and social care systems. Complexity of the rare disease means that patient health needs cut across disciplines and there is a lack of multidisciplinarity (or multidisciplinary teams) working (in reality) and a lack of holistic care. Greater integration of health and social care has been shown to directly reduce these inequalities.

Health inequalities show a significant discrepancy across Europe with a dramatic variation in survival rates due to accessing trained, experienced medical team. These inequalities can disproportionately affect rural populations due to limited access to services and further

compounded by the lack of willingness to accept a PLWRD in everyday life. Clinical networks and tele-expertise have started to test how to reduce crossborder inequalities.

**Chaired by:**
**Maria Montefusco**, Board of Directors, EURORDIS-Rare Diseases Europe

**Speakers:**
**Dr Julia Faulkner,** Patient Advocate, Tracheo-Oesophageal Fistula Support (TOFS) UK and EAT
**Encarna Guillén- Navarro**, Hospital Clinico Universitario Virgen De La Arrixaca, Unit for Medical Genetics
**Tony Holland,** President, International Prader-Wili Syndrome Organisation (IPWSO)
**Dorica Dan,** President, Romanian National Alliance for Rare Diseases (RONARD) and Board of Directors member, EURORDIS
**Isabella Brambilla,** Member and President, Dravet Italia Onlus
**Prof. Wout Feitz,** ERN eUROGEN HCP Network Coordinator
**Andreas D. Rosenberger,** Centre Leader, National Neuromuscular Centre, Norway

**Track D Session 2:** Wednesday 29 June 2022, 16:30 – 18:00 CET

## Inequalities in accessing education, work and independent living for people living with a rare disease.

To have the same opportunities for economic and social independence, access employment is predicated on acquiring the necessary skills and abilities from school and the education system. Educational systems and schools are not always effective in responding and adapting to the physical and emotional health needs of someone with a complex rare disease, without creating equalities for the individual in accessing the same opportunities in social and educational activities as others. Even with a solid education and having the capacity and desire to work, people living with a rare disease have can face serious stigma and discrimination leading to increased challenges to access employment or returning to work following treatment. This is also the case of their carers who need support to fulfil their caring role whilst remaining in employment.

This session will explore the everyday challenges for a PLWRD to access education and employment and sharing good practice of how schools and business can be inclusive and offer an individual with the same opportunities to contribute to society and be independent. Many PLWRD experiencing the disability gap widening and increased social isolation and stigma in adulthood. The session will also explore societal normal and public perceptions

influence inclusion in employment and society, sharing good practice to inform a policy discussion.

**Chaired by:**
**Matt Bolz-Johnson**, ERN & Healthcare Advisor, EURORDIS-Rare Diseases Europe.

**Speakers:**
**Allison Watson,** Chief Executive Officer and Cofounder, Ring20,
**Oscar Raul Lozano,** University of Valencia
**Verity Fisher,** Physiotherapy Manager, National Star
**Jamie Bolling,** Research Developer, Swedish Disability Federation
**Petia Stratieva,** Retina International
**Loredana Dicsi**, Membership, Internal Communication & Youth Officer, European Disability Forum

# INDUSTRY, INNOVATION AND INFRASTRUCTURE

**GOAL LEADERS:**

**Ruxandra Draghia-Akli**, The Janssen Pharmaceutical Companies of Johnson and Johnson

**Simone Boselli**, EURORDIS

**Hans-Georg Eichler**, Association of Austrian Social Security Bodies

**Virginie Hivert**, EURORDIS

**With the support of:**

**Maurizio Scarpa**, MetabERN

**Anton Ussi**, EATRIS

## Goal description

Track E will address the challenge of building a cohesive rare disease health data ecosystem echoing the resilient infrastructure mentioned in SDG 9.

"Within the next 10 years, outcomes, actionable data should be routinely used to accelerate diagnosis, to guide care and management, to support better coordination of care, drug development, HTA and regulatory decision-making and inform health policy."

This vision for a seamless European rare disease health data ecosystem, as described in the Rare2030 Recommendations, envisages a cohesive data landscape where epidemiological, healthcare, research, quality of life and treatment-related data are shared and linked at a European and global level where possible. Sharing of data for different purposes is optimised across infrastructures and countries, relying upon commonly adopted codification systems (Orphanet nomenclature), harmonised standards and interoperability requirements. Under this vision, national data ecosystems are linked seamlessly to the European ecosystem via Findable, Accessible, Interoperable and Reusable (FAIR) data approaches.

Such an ecosystem, centred on robust European Reference Networks (ERNs) and by extension their clinical research capacities, the European Platform on Rare Disease Registration, and other key infrastructures would be well anchored within the European Health Data Space (EHDS), benefitting from this new framework for data sharing, while also contributing to build this space in a way that acknowledges the specificities of rare disease data and is responsive to the needs and expectations of the rare disease community.

So, how can we transform this vision into reality? Is the rare disease community ready to tap into the opportunity that the EHDS represents to address the challenges faced by people living with a rare disease? The answer: not yet.

Using the health data journey as a compass to structure the discussion, these two sessions will help to disentangle the myriad of initiatives and facilitate an honest discussion among stakeholders to identify what data are being collected; when and for what purposes each stakeholder needs to use the data and what would be acceptable conditions to share the health data sets that each is collecting and curating.
How can we make sure that data collected and used upstream (e.g. research/diagnosis/care) can be re-used downstream (e.g. to improve the quality of care/therapeutic development/regulatory process/policy and HTA decision-making) and vice-versa, and how can this be orchestrated? What are the immediate measures to set the foundations of a cohesive European health data ecosystem for rare diseases that can benefit from and contribute to the larger EHDS?

Track F will address mainly the second part of SDG 9, hence innovation, Europe's attractiveness for investment and medicine development, as well as sustainability. The two main objectives are as follows:

- How can we keep Europe attractive for development of treatments and interventions for people living with a rare disease?
- Can Europe be attractive and sustainable at the same time?

To approach these questions, we will look at what is needed to fix framework issues if we continue to operate within the current ecosystem for treatment and interventions development and access for patients. What choices could we make if we would like to address the needs of the entire society based on solidarity and equity, as called upon by the Rare 2030 recommendations?

When looking at the medicine lifecycle from development to access, points to be tackled can be easily identified. The first is the so-called 'valley of death' which exists between the basic research and early stage of development, and the late stage of clinical trials. Indeed, this 'valley of death' is not only problematic in the field of rare diseases, but specific issues and ways of de-risking product development in rare diseases are acute and need to be urgently addressed. A major obstacle is linked to the challenges of clinical trials in small patient populations, on a methodological, operational and/or a financial level. This brings into perspective the importance of the Directive on Patients' Rights in Cross-Border Healthcare (CBHC), the directive for clinical trials and the various funding mechanisms (angel and private investor funds, public, private, and/or public-private partnerships and funding sources). By no means least is the fragmentation of the EU market: products are granted centralized European Marketing Authorisation, but pricing and reimbursement decisions taken at a national or regional level lead to inequalities in access and availability. The current revision of the EU Orphan Medicinal Product (OMP) regulation and of the general pharmaceutical framework is paving the way for some of these issues to be solved by the legislators, but it cannot fix everything. Innovative solutions must also be put forward on the R&D and access fronts in order to unlock the potential of currently disregarded diseases, to ensure the continuity of evidence generation and to allow the defragmentation of the marketplace, i.e. new models of collaboration and funding.

On top of these improvements, we need to reflect on what kind of society we want to live in and how people living with a rare disease want to see the society evolve while keeping rare disease at the top of the policy agenda. Building on the Rare 2030 recommendations and on the principles of solidarity, equity and social justice, what political choices are needed to combine the EU's attractiveness for companies - ensuring that Europe is a good place for investment and innovation - and the resilience of the healthcare systems for orphan drugs expenditure, reduction of inequalities between countries and within rare diseases? How do we integrate effectively into our society the ground-breaking innovation that tackle the genetic root causes of the great majority of rare diseases?

The central question is: how can we align values across the development chain? From the decision maker's point of view, what criteria should preside within the distribution of resources for healthcare budgets; how do payers become buyers? On the developer's side, where do we stand with regards to ESG (Environmental, Social and Governmental) approaches in view of fulfilling the SDGs? What are the different options to work upstream on assets, fair price, value-based pricing, procurement, sustainable bonds and so on? What would it take to switch from profit-only-based approaches to approaches which bring societal and environmental benefits? Is B-Corp (or Benefit corporations) the way to go for companies? Should there be this type of indicator attached to this value-based decision making on pricing and reimbursement? These are the questions that we would like to discuss with you all!

## Track E - The health data challenge: Building a Cohesive Rare Disease Health Data Ecosystem

**Session 1:** Thursday 30th June 2022, 14:00 – 15:30 CET

### Building a seamless health data ecosystem

This session will provide a state of the art of the needs of rare diseases in the European Health Data Space, where are we now, where are we going and where do we want to go? How do we connect data to the wider eco-system to allow those who need it to be able to find it and access it when required? This session will explore the specific challenges and opportunities for the rare disease community. How can we orchestrate exchange and what key immediate actions are needed to build a seamless health data ecosystem?
For this, we will get a clear view of the roadmap that is designed in the EHDS (definition, scope, boundaries and timelines) and reflect as well on how it relates to the Members States level. We will then hear from the current pilot that is put in place for rare diseases and see which opportunities it brings for the whole community. Finally, we will get inspired by the COVID situation which seems to show us that the issues are not technical and that when there is willingness and budget, nothing is impossible - as per the subtitle of our conference, mission …possible.

**Chaired by**
**Mark Hanauer,** Co-Director and Director of Innovation Strategy, Orphanet

**Speakers**: To be confirmed

---

**Session 2 :** Thursday 30th June 2022, 16:00 – 17:30 CET

### How to make the best use of ERNs & improve the efficiency of the whole system

This session will cover how we generate data, the amount of data generated, for what purposes and for whom. Some key questions are: How can we make better use of data to advance research on rare diseases? How to better enable real-world data (RWD) for legitimate research and decision making? How does GDPR affect research on rare diseases?

In this session, we will try to find answers to these questions while also debunking some myths related to data sharing. Different practical examples and initiatives on health data and real-world evidence will be presented. We'll identify the points of discussion, map actions needed and prioritise actions to build from.

The speakers are clinicians/researchers who are working hand-on with data. They will share their experience on how to generate data of good quality, how to organise the data collection better in the context of diagnosis, epidemiology, natural history and more. We also hear what conditions are needed to use or re-use the data, what types of data handling are needed, how the machines use the data (machine learning, AI, natural languages processes, algorithms, keywords). And finally what does that mean for healthcare professionals, how do we offer tutoring and training for the medical professionals and prepare for the digital doctors of 2030?

**Chaired by:**
**Maurizio Scarpa,** Coordinator of the European Reference Network for Hereditary Metabolic Diseases, MetabERN **.**

**Speakers:**
**Prof. Ronald Cornet,** Associate Professor, Department of Medical Informatics at Amsterdam Public Health Research Institute
**Dr Sergi Beltran,** Head of Bioinformatics Unit and Data Analysis Team at Centro Nacional de Análisis Genómico
**Dr Francesco Cremonesi,** Technical Lead, Datawizard
**Prof. Ronan Fleming,** Associate Professor, National University of Ireland, Galway, & Assistant Professor, Leiden University

## Track F: Building resilient infrastructure, promoting inclusive and sustainable industry and fostering innovation for people living with a rare disease

**Session 1:** Thursday 30th June 2022, 14:00 – 15:30 CET

## Making Europe attractive for therapies development for PLWRD- addressing the valley of death

This session will focus on addressing the 'technical efficiency' issues that exist in Europe, focusing specifically on the ongoing revision of the incentives framework, the implementation of the new regulation on HTA cooperation in Europe, and the possibility for more equitable access to authorised therapies provided by new models.
This session will be formatted as a series of three short moderated conversations between speakers to break down the opportunities and challenges laying ahead of us, preceded by an opening introduction by the Chair.

**Chaired by:**
**Ruxandra Draghia Akli,** Global Head, Global Public Health R&D at The Janssen Pharmaceutical Companies of Johnson & Johnson

**Speakers:**
**Alexander Natz,** Secretary General, European Confederation of Pharmaceutical Entrepreneurs
**Olga Solomon,** Head of Unit, Medicines: policy, authorisation and monitoring unit, European Commission - DG Health and Food Safety
**Dr Alicia Granados,** Head, Global HTA Scientific Strategy, Sanofi Genzyme
**François Houÿez,** Director of Treatment information and Access, EURORDIS
**Nathalie Moll,** European Federation of Pharmaceutical Industries and Associations, Director General
**Avril Daly,** EURORDIS Board Member, CEO of Retina International
**Tamsin Rose**, Senior Fellow for Health, Friends of Europe

---

**Session 2:** Thursday 30th June 2022, 16:00 – 17:30 CET

## Can Europe be attractive and sustainable at the same time?

This session will focus on addressing the 'allocative efficiency' or on how to distribute resources to rare diseases therapies within budgets. Furthermore, this session will look at the maturing concepts that are gaining momentum to ensure sustainability and affordability in Europe through ethical approaches (ESG indicators, benefit corporation, social pacts, etc.)
This session will begin with an introduction to the topic by the chair (Hans Georg Eichler) followed by a series of short presentations to highlight the concepts and progresses made in integrating new ways to address sustainability and accessibility of therapies for rare diseases

**Chaired by**
**Hans-Georg Eichler,** Consultant Physician, Association of Austrian Social Insurance Institutions

**Speakers:**
**Giacomo Chiesi,** Head of Global Rare Diseases at the Chiesi Group
**Yann Le Cam,** CEO, EURORDIS
**Momir Radulovic,** Executive Director, Agency for Medicinal Products and Medical Devices of the Republic of Slovenia
**Kasha Witkos,** Senior Vice President and Head of International, Alnylam
**Sarah Garner,** Senior Policy Advisor, Access to Medicines and Health Products, WHO